

# **New Medicines Reviews 2013**

## FOREWORD



The human body's ability to fight disease and recover from illness is nothing short of remarkable, just as its fragility in the face of the ravages of many diseases and conditions can be heart-breaking. It is this fundamentally human desire to prevent these ravages and to save or extend life that is at the very core of the medical science of pharmacology.

Developing, understanding and utilising the complex chemicals or biological molecules calls on a range of expertise to ensure that right therapeutics are used effectively in the right clinical situation.

Building on the many years of collective experience of NHS clinicians the processes such as Area Drug and Therapeutic Committees (ADTCs) and the Scottish Medicines Consortium (SMC) have evolved to ensure that medicines both efficacious and represent value for money are recommended for use across NHSScotland so that people across Scotland can benefit as quickly as possible., Where clinicians believe there is a case to prescribe a medicine which has not been recommended, they are able to instigate an Individual Patient Treatment Request (IPTR).

However, following some concerns expressed by clinicians and patient representatives regarding some aspects of SMC, ADTC, IPTR processes, I was clear that it was time for a full expert review of every aspect of the assessment, introduction and availability of new medicines, to ensure that these processes enable medicines to make the maximum contribution to creating the safe, person-centred and effective healthcare we are committed to providing for everyone, as set out in my 2020 vision for health and care in Scotland.

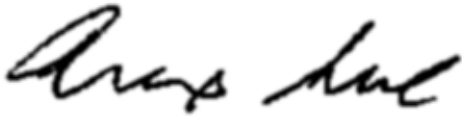
Professor Routledge was commissioned to review the current new medicines assessment procedures of the SMC and make any recommendations to improve the process. Professor Charles Swainson was similarly commissioned to review how SMC accepted medicines as introduced into ADTC formularies, to review both the ADTC network and IPTR's and to make recommendations.

The reviews have now been received and their recommendations include:

- More public involvement within SMC.
- Increased flexibility in the consideration of new evidence on cost or effectiveness of a drug while it is being assessed.
- More public involvement in Health Board ADTCs and the IPTR processes.
- Health board decisions on IPTRs should be published making them more open and transparent.
- Increased scrutiny and standards should be put in place to continually review requests for drugs not recommended for routine use.

An interim recommendation from the review - a £21 million fund to cover the cost of medicines for patients with very rare conditions - has already been introduced.

I am grateful to Professor Routledge and Professor Swainson for the rigour, thought and time they have given to produce their reports. I will carefully consider their recommendations alongside the conclusions that the Health and Sport Committee will reach when it completes their report, and the responses from the external consultation process.

A handwritten signature in black ink, appearing to read 'Alex Neil', is enclosed within a thin black rectangular border.

**Alex Neil MSP**

Cabinet Secretary for Health and Wellbeing etc.

**THE AVAILABILITY OF NEW  
MEDICINES FOR PATIENTS IN  
SCOTLAND:**

**The role of the Scottish Medicines  
Consortium (SMC)**

**April 2013**

# Contents

<b>Summary</b>	5
<b>Background</b>	
1. - Introduction	7
2. - Remit of the Review	9
<b>The Process for medicines appraisal</b>	
3- The Present Process in Scotland	10
4- Comments received from consultees	15
5. – Findings	18
<b>A way forward</b>	
6. Recommendations	23
<b>Appendices</b>	
References	28
Glossary of terms and abbreviations	31
List of Consultees	34

## Summary

Many medicines provide major benefits by enabling patients with a wide range of illnesses to live longer and/ or experience a better quality of life. While many of the new medicines which are marketed each year are available to all NHS patients in the UK, some are not. Medicines appraisal, the process by which the NHS in Scotland decides whether the benefit to patients justifies any additional cost in their purchase, is conducted by the Scottish Medicines Consortium (SMC).

Since 2001, SMC has been tasked with appraising all medicines launched on the UK market in a timely, efficient, robust and independent fashion, an achievement of which health professionals in Scotland are quite justifiably proud, and a process in which they have confidence. Comparisons with NICE have shown that while the proportion of medicines recommended for NHS use are similar to NICE, SMC publishes advice more quickly (*Dear et al 2007, Barbieri et al 2009, Ford et al 2012*). Their advice is conveyed to Area Drug and Therapeutics Committees (ADTCs) in the Health Boards, who ultimately decide upon the implementation of this advice.

SMC performs the difficult task of helping the NHS in Scotland to obtain value for money from those medicines which have recently obtained their marketing authorisation in the UK/Europe. For the last 12 years, it has fulfilled this challenging role by developing a fast, efficient, robust and independent process that has developed and adapted to changing needs and new circumstances. Its procedures are based firmly on the principles of evidence based medicine (EBM) and health economics, using rigorous and widely-accepted methodologies.

It is clear that the service which SMC provides is highly regarded and greatly appreciated by many health professionals working in the NHS in Scotland. Indeed SMC and its processes are held in high esteem internationally, and other HTA bodies worldwide have learned much from SMC when establishing their own systems. Others have compared their decision-making processes with those of SMC (*Lexchin and Mintzes 2008, Bending et al 2012*). The SMC process draws heavily on the expertise in evidence based medicine and health economics available in NHS Scotland and its two-stage process (assessment at New Drugs Committee [NDC] and then appraisal at SMC) adds further rigour and in-depth consideration. Examination of the documents made available to us and our face-to-face discussions with staff confirm the quality of the work leading up to preparation of the evidence for consideration at NDC and then SMC. NHS Scotland has developed a Patient Access Scheme (PAS) process which has further improved access to medicines by achieving greater value for money. This has been a result of the close engagement with the pharmaceutical industry from the inception of SMC.

It is vital that SMC maintains its well-earned international reputation for excellence, and continues to adapt to the expectations of the public, patients and those who submit their medicines for scrutiny. To do this, its processes should continue to evolve, as they already have over the last decade, and consideration should be given to the following recommendations:

**Recommendation 1. SMC should meet in public so that members of the public, patients, patient group representatives, other health professionals and members of the pharmaceutical industry can attend to observe the appraisal process.**

**Recommendation 2. SMC should invite the manufacturer of the new medicine under consideration to give evidence at their main SMC appraisal meeting, in order to address any outstanding questions that SMC members have and highlight any outstanding issues of which they believe SMC should be aware prior to its advice being published.**

**Recommendation 3. SMC should be able to appraise any new medicines which the NHS in Scotland considers potentially of major importance to patient care, but which have not been submitted to SMC by the manufacturer within 12 weeks of launch. If necessary this appraisal may be conducted using such data as is already available in the public domain.**

**Recommendation 4. SMC should be able to have a temporary pause in the appraisal process at any stage in order to permit further dialogue with manufacturers on issues that would be likely to be central to the subsequent decision-making process.**

**Recommendation 5. SMC should develop a policy specifically relating to ultra-orphan medicines to guide the process of consideration of all available evidence relevant to its advice on these medicines.**

**Recommendation 6. SMC, with the appropriate resource and in partnership with other relevant bodies in Scotland, should be encouraged to set up an engagement process such as a “Citizen’s Council” or “Citizen’s Jury” to explore views around specific societal issues of importance to the people of Scotland in relation to the availability of new medicines and the impact of these views on the existing processes for ensuring access to medicines.**

**Recommendation 7. SMC should explore other innovative approaches to increasing patient and public awareness of its role in ensuring timely access to clinically effective and cost-effective medicines in Scotland. Consideration should also be given to expansion of its role to support other aspects of safe, effective and cost-effective prescribing. SMC should produce a publicly available annual report of progress in this regard detailing its important contributions to this objective.**

**Recommendation 8. NHS Scotland should explore ways in which the expertise available within SMC might be used to support the process of Value Based Pricing (VBP).**

**Recommendation 9. A register of IPTR decisions in all Health Boards, suitably anonymised to protect patient confidentiality should be kept, and supporting information related to IPTRs shared between Health Boards.**

**Recommendation 10. There should be regular sharing of experiences between the IPTR panels and members of IPTR panel members across Scotland should meet at least annually for induction, feedback and training.**

# Background

## 1. Introduction

**1.1.** Medicines are vital therapeutic tools in modern healthcare, controlling many conditions and in some conditions, achieving cures. Every year, approximately 70 new medicines or new formulations receive a Marketing Authorisation (MA) from the European Medicines Agency (EMA) and the NHS must then decide whether the additional cost in purchasing them is justified by the likely benefit to patients. This process is termed *medicines appraisal*, a specific type of health technology appraisal (HTA), and central processes for this activity have existed in Scotland, England and Wales for over ten years in the Scottish Medicines Consortium (SMC), National Institute for Health and Clinical Excellence (NICE) and the All Wales Medicines Strategy Group (AWMSG), respectively.

**1.2.** SMC is a national collaboration between the Area Drug and Therapeutics Committees (ADTCs) in the Scottish Health Boards to reduce duplication and improve consistency of advice. Its subcommittee, the New Drugs Committee (NDC), and SMC itself meet monthly in Glasgow. It first began its work in 2001, under the chairmanship of Professor David Lawson. Since that time, it has conducted appraisals of individual newly licensed medicines (licensed from January 2002), (including new indications for medicines with an existing license) on behalf of the Scottish Health Boards. It also considers new formulations of existing medicines. It does not appraise vaccines, branded generic medicines, blood products or diagnostic drugs. Device-containing medicines are appraised if they are licensed as medicines by the MHRA/EMA.

**1.3.** The process employed by SMC is often termed a “single technology assessment” or “single technology appraisal” (STA). SMC’s role is to recommend for routine use, those newly licensed medicines which it considers represent good value for money for NHS Scotland. The manufacturer submits a clinical and economic case, which is first considered by the New Drugs Committee (NDC), a sub committee of SMC (see later) and then by the main Committee (SMC). The recommendations are then forwarded to the ADTCs in the Scottish Health Boards.

**1.4.** The ideal medicines appraisal process should be *transparent, timely, relevant, in-depth* and *usable* (Cox Report, cited by Garrido et al 2008). *Transparency* of the process is important if disagreements about the process itself rather than scientific issues are not to predominate. It also encourages greater trust in the HTA body and its processes. It requires full declaration of interests over the appropriate period of time by participants. *Timeliness* ensures that medicines which are both clinically effective and cost effective can be made available to patients as soon as possible after launch. *Relevance* is important in ensuring that the advice produced is appropriate and applicable to the needs of the user and therefore *usable*. The HTA process needs to be *in-depth*, to ensure that it is as robust and reliable as possible. In addition, there are other important attributes of the ideal process. Advice needs to be *accessible* (a large number of pieces of advice available in common format on a database that is easy to navigate). The process should be *efficient* (value for money



and avoidance of duplication of effort). Scientific *Independence* of the organisation conducting *the HTA process* from interested parties, including policy-makers and government is vital (*Garrido et al 2008*) and ensures that the advice produced is accepted by health professionals as well as patients, the public and pharmaceutical manufacturers.

**1.5.** SMC aims to provide a decision on all new medicines within three to four months of launch, so that early access to new medicines is possible for Scottish patients (*Kohli 2005, Cairns 2006, Webb et al 2006, Dear et al 2007*). SMC is the only HTA body to consider STAs of all new medicines. AWMSG in Wales reviews those medicines not on the NICE work programme and NICE appraises only selected medicines referred to it by the Department of Health. SMC's advice is published rapidly (*Dear et al 2007, Ford et al 2012*) and used and referenced internationally (*OFT 2007, Lexchin et al 2008, Vegter et al 2010*). SMC, like AWMSG, only appraises medicines whilst NICE also considers selected healthcare interventions. SMC and NICE recommend a similar proportion of medicines (*Ford et al 2012*),

**1.6.** The standards achieved by SMC and the other UK HTA bodies were highlighted in a report by the Office of Fair Trading (OFT) on the Pharmaceutical Price Regulation Scheme (PPRS) in 2007. Discussing the role of NICE, the SMC and AWMSG it noted that, "The technical expertise that these bodies bring to bear in conducting cost effectiveness assessments is of world class standard." It also stated that they have made a significant contribution to the cost-effective use of NHS resources and have "shown themselves able to adapt to changing needs" (*Office of Fair Trading 2007*). Specifically in relation to SMC, the report commented that "SMC decisions are highly regarded and the body has managed to establish a strongly collaborative approach with industry throughout its evaluation process"

**1.7** Despite SMC's progress in adapting to changing needs in Scotland over the last decade, there have been recent concerns expressed by some clinicians, charities and pharmaceutical industry about difficulties in patients being able to access effective medicines in Scotland, prompting the decision to institute a review of SMC processes and access arrangements.

## 2. Remit of the review

### 2.1. New Medicines Review

The New Medicines Review announced by the Cabinet Secretary for Health and Wellbeing on the 14 November 2012 responds to concerns raised in the Scottish Parliament and the media regarding concerns of patients, clinicians and other stakeholders regarding access to new medicines – particularly cancer medicines and medicines to treat rare diseases.

The New Medicines Review will examine every aspect of the processes for introducing new medicines within the NHS in Scotland to assess their effectiveness and to identify what further improvements can be made.

The review comprises three main strands of work:

- (i) an independent examination of the Scottish Medicines Consortium (SMC) appraisal processes; **(Annex A)**;
- (ii) an examination of the role and remit of NHS Board Area Drug and Therapeutics Committees (ADTCs) and a separate examination of the current Individual Patient Treatment Request (IPTR) arrangements **(Annex B)**; and
- (iii) An audit of NHS Board formulary decisions regarding medicines which have been accepted or accepted for restricted use by the SMC (Annex C).

A key aim of the review will be to achieve consistency in the application of national policy throughout NHS Scotland. A fuller description and the scope of each strand of work are set out in the attached annexes.

**2.2.** This report aims to address the first of these three strands, an independent examination of the Scottish Medicines Consortium (SMC) appraisal processes.

**2.3.** Annex A (below) outlines the terms of reference of that particular strand:

#### **Annex A: Description and Scope**

Professor Philip Routledge, Professor of Clinical Pharmacology at Cardiff University and Mrs Karen Samuels, Head of HTA and Medicines Management in the All Wales Therapeutics and Toxicology Centre, Cardiff, Wales will examine the current Scottish Medicines Consortium (SMC) appraisal processes and methodology from horizon scanning of new pharmaceutical products in the pipeline through to provision of SMC advice to NHS Scotland to see what further improvements can be made.

This will include a broader view of the cost and benefits of new, innovative medicines – specifically to assess whether the Quality Adjusted Life Year (QALY) methodology represents an effective tool to calculate cost-effectiveness of these in terms of off-setting the cost of the medicine against potential savings.

# The Process for Medicines appraisal

## 3. The present process in Scotland

**3.1.** The Scottish Medicines Consortium (SMC) was established in 2001 to provide advice to NHS Boards and their Area Drug and Therapeutics Committees (ADTCs) in Scotland concerning the status of all newly licensed medicines, all new formulations of medicines already licensed, and new indications for established medicines (licensed from January 2002) as close to launch as possible. Its first advice was produced in April 2002 (Hems et al 2012). It meets monthly on the first Tuesday of the month. SMC does not assess vaccines, branded generics, blood products, and diagnostic drugs. SMC is a consortium of NHS Scotland's 14 Health Boards.

It also provides a horizon scanning service so that NHS Boards can be aware of new medicines in the drug development process and thus plan their budgets more effectively.

**3.2.** The membership of SMC (and its subcommittee, the New Drugs Committee [NDC]) consists of staff (health professionals and senior managers) from the Area Drug and Therapeutics Committees (ADTCs) in Scotland who serve for three years in the role. On SMC, there are also two representatives from the Association of British Pharmaceutical Industries (ABPI) and lay representatives, termed "public partners" who are appointed after interview. The full membership of the main committee is around 40 and that of the NDC around 20. The current membership is available on the SMC website ([www.scottishmedicines.org.uk](http://www.scottishmedicines.org.uk)). Meetings are accepted as quorate with an attendance of 50% plus one voting member (i.e. excluding attendees or observers).

Both committees meet monthly. The Committees are supported in their deliberations by pharmacists experienced in critical appraisal and by health economists. Neither the NDC nor SMC meeting is held in public. All members are required to adhere to stringent requirements to declare relevant conflicts of interests in the manufacturers of the medicines being appraised, or in manufacturers producing competitor products.

**3.3.** The New Drugs Committee conduct a rapid assessment of the submissions from pharmaceutical manufacturers and then make recommendations to SMC concerning the clinical effectiveness and cost effectiveness of the medicine. Advice is generally arrived at by consensus, but a simple majority vote by full members of NDC (and SMC) can be held when necessary. NHS Boards are expected to follow SMC advice, but because most medicines appraised by SMC are for indications for which there are alternative treatments, the implementation of SMC accepted medicines is subject to local NHS Board decision. The Board can decide whether or not it will include the SMC accepted medicine in its formulary by reviewing the medicine in the light of other comparable medicines already available within the Board formulary or approved list for the same indication.

**3.4.** In a **full submission**, manufacturers are required to demonstrate that the medicine will either “provide additional health benefits that are valued by patients compared to current Scottish practice and that this is at a net cost to the NHS that offers acceptable value in relation to other uses of the same resources,” or “offers equivalent levels of health benefit to patients at an equivalent or lower net cost to the NHS”. (*Anon. Working with SMC – A Guide for Manufacturers*). The submissions are presented in a structured template to achieve efficiency and enhance the speed of the process, and consistency and comprehensiveness in information submitted. If the manufacturer wishes to make the case for use of the medicine only in one group within the licensed indication and not for the whole group represented by the licensed indications, a **selective submission** is possible. It is also sometimes referred to as ‘niced’ submission, and requires that the manufacturer should ensure that the proposed population for treatment is appropriate and valid within the licensed indication under consideration in the submission.

**3.5.** An **abbreviated submission** may be considered in certain circumstances e.g. for a new formulation of an established product, or for a medicine that has previously been accepted by SMC when the marketing authorisation is subsequently extended to include use in children or adolescents.

**3.6.** SMC may accept a medicine for use without restriction. In such case guidance from Scottish Government is that “there is a clear expectation that NHS Boards will consider it and will make it (or its equivalent) available” (*Scottish Government 2012*). It may sometimes recommend restricted use. The restriction may be for a more limited indication or patient population than the licensed indication. SMC can also issue advice that the product should not be recommended for use in Scotland. Manufacturers can resubmit to SMC on the basis of new scientific information, and also have the option to ask for an independent review of the decision. An Independent Review Panel then reconsiders the originally submitted evidence and reports its findings to SMC. If SMC issues “not recommended” advice in relation to a particular medicine, NHS Boards do not have to make it routinely available. Nevertheless, medicines “not recommended” by SMC, including those medicines “not recommended” due to a non-submission, may be made available under certain circumstances through individual patient treatment requests (IPTRs).

Recent guidance from the Chief Medical Officer for Scotland outlines good practice guidance for IPTRs (*Chief Medical Officer 2011*). It states that they should not be used to circumvent established assessment processes. It also notes that when SMC / NHS QIS advice is still being awaited, the policy position across Scotland is that it would not be expected that such a medicine would be routinely prescribed. The guidance does acknowledge that in such circumstances, NHS Boards may wish to consider IPTRs “where the clinician responsible for the patient believes a delay in treatment pending SMC/NHS QIS advice would result in a significant adverse outcome for the patient”.

**3.7.** SMC, like NICE and AWMSG, bases its advice on an assessment of the cost of the medicine and the clinical benefits (likely extension of life and improvements in the quality of life) and these are commonly expressed in terms of the cost of the medicine per Quality-Adjusted Life Year (QALYs) gained. Although SMC does not have a formal cost effectiveness threshold, like NICE and AWMSG, it generally recommends clinically effective new medicines when the incremental cost

effectiveness ratio or ICER (incremental costs and benefits associated with the new medicine compared to those of an appropriate existing comparator) is lower than £20,000. However the cost per QALY is only one factor informing SMC's broader judgement of the value of a new medicine (see 3.8).

**3.8.** Additional factors termed “modifiers” may be used to allow greater flexibility in certain circumstances. This allows SMC to accept greater uncertainty in the health economic case. In some cases it allows acceptance of or a higher cost per QALY. There are specific modifiers that apply to orphan drugs, with this specific EMA designation.

Some of the modifiers listed by SMC as potentially relevant in some cases (e.g. when the cost per QALY is high) are:

- Evidence of a substantial improvement in life expectancy (with sufficient quality of life to make the extra survival desirable). Substantial improvement in life expectancy would normally be a median gain of 3 months but the SMC assesses the particular clinical context in reaching its decision;
- Evidence of a substantial improvement in quality of life (with or without survival benefit);
- Evidence that a sub-group of patients may derive specific or extra benefit and that the medicine in question can, in practice, be targeted at this sub-group;
- Absence of other therapeutic options of proven benefit for the disease in question and provided by the NHS;
- Possible bridging to another definitive therapy (e.g. bone marrow transplantation or curative surgery) in a defined proportion of patients;
- Emergence of a licensed medicine as an alternative to an unlicensed product that is established in clinical practice in NHS Scotland as the only therapeutic option for a specific indication

**3.9.** SMC recognises the category of “orphan drug” using the EMA definition – “one licensed for treating or preventing life-threatening rare diseases affecting fewer than 5 in 10,000 people in the European Union”. During the appraisal process, SMC is prepared to accept a greater level of uncertainty in the calculation of cost effectiveness and a higher calculated QALY (when supported by a robust clinical and economic case) if other additional factors are present, namely whether:-

- the drug treats a life threatening disease
- whether it substantially increases life expectancy and/or quality of life
- it can reverse, rather than stabilise, the condition
- or bridges a gap to a “definitive” therapy,

This list is not exhaustive, however.

SMC does not recognise the category of medicines for extremely rare conditions known as ultra-orphan medicines and so has no specific policy for their appraisal, although they clearly still fall into the EMA and (therefore SMC) orphan drug category. Ultra-orphan medicines were first separately identified by the Citizen's Council of NICE as those medicines licensed for the treatment of diseases with a UK

prevalence of less than 1 in 50,000. This equates to around 100 prevalent cases in Scotland (NICE 2004). The low prevalence means that manufacturers of these medicines find it difficult to recoup the costs incurred in developing the drug and obtaining a marketing authorisation. The cost per patient is therefore generally very high with the effects that these medicines may not reach conventional “thresholds” for cost-effectiveness.

**3.10.** NHS Scotland accepts Patient Access Schemes proposed by the manufacturer of the medicine to improve the cost-effectiveness of medicines, so that if also deemed clinically effective, it is more likely to be approved by SMC and therefore patients will be able to access the medicine. NHS Scotland consider these schemes via a national body separate from SMC, The Patient Access Scheme Assessment Group (PASAG) consider those medicines:-

- (i) that are not, or might not be, in the first instance found to be cost-effective by SMC

or

- (ii) where a patient access scheme has been accepted in the context of a NICE Multiple Technology Appraisal (MTA). All PAS submitted with a NICE MTA must be evaluated for acceptability in Scotland by PASAG and the MTA endorsed by Healthcare Improvement Scotland (HIS)

**3.11.** SMC has established a Patient and Public Involvement Group (PAPIG), chaired by a Public Partner, to support and advise patient interest groups to make submissions to inform the HTA process. The stated aims of the group are listed on the SMC website and are to make recommendations to SMC on the development of public involvement opportunities; to monitor and report back to SMC on the effectiveness of public involvement opportunities where this information is available; to ensure that a patient/ carer perspective is prominent in all SMC assessments; to present a summary of the Patient Interest Group submissions, where these have been submitted to SMC meetings and to promote public awareness of, and involvement in, the work of SMC.

A report of the work of PAPIG in encouraging patient interest group involvement in SMC’s HTA programme was published annually in the SMC annual report between 2001-02 and 2008.

**3.12** Dialogue with the ABPI occurs via the User Group Forum (SMC UGF). SMC UGF consists of individuals representing the pharmaceutical industry together with selected members of SMC and NDC. Its role is to identify and resolve any issues relating to SMC’s process for HTA.

**3.13.** In addition to its HTA role, SMC provides a horizon scanning service to named individuals within Health Boards who are involved in financial planning. This supports service planning, and is provided to key senior Health Board staff, after they have signed a confidentiality agreement, since some of the information is commercial-in-confidence. An annual report “Forward Look” has been produced each year since 2003. It projects estimates of the uptake of medicines, and the potential resultant net budget impact. This takes into account any likely replacement of existing medicines,

as well as any estimated additional costs or savings associated with diagnostic testing, delivery and monitoring of the medicine.

**3.14.** SMC also hosts the Scottish Antimicrobial Prescribing Group (SAPG), a national clinical multi-disciplinary forum to support antimicrobial stewardship by promoting rational, safe and effective antimicrobial prescribing use in NHS Scotland, both in primary and secondary care.

## 4. Comments received

Written comments were received from a range of organisations, including Patient Interest Groups, pharmaceutical manufacturers and health professional groups. In addition, around 25 individuals were interviewed, either separately or in groups, either face to face or by telephone. This section condenses the views received from those consultees in relation to SMC's appraisal process. Several comments were also received concerning the IPTR process. While not within the direct remit of this part of the review, we recognise that SMC's judgements have an impact on the IPTR process, so we have included some general comments, and have made two recommendations in this area.

### 4.1 Patient Interest groups

In the evidence submitted by patient interest groups, there was acknowledgement that all new medicines need to be evaluated for clinical effectiveness. There was also acknowledgement that the work of SMC was rigorous, of high quality and rapid. There was also a request that the term ultra-orphan medicine be recognised and a request that consideration should be given to the development of a separate process for assessing ultra-orphan drugs. One suggestion was that the appropriateness of a separate body to assess orphan and ultra-orphan indications, similar to the Advisory Group for National Specialised Services (AGNSS) which operates in England might be considered.

Greater and more meaningful patient involvement was called for. A specific option suggested was that that individuals as well as organisations might be able to feed into the process. Feedback from SMC about the value and impact of the submissions would also be welcomed, so that patients who had spent time sharing their experiences for the submission could be assured that their contribution had been fully considered by SMC. There was also a call for a more transparent working process to foster trust, understanding and openness, with opportunities for a patient or patient representative to attend the meeting of SMC.

### 4.2. Pharmaceutical Industry views.

There was an acknowledgement that the SMC process resulted in fast and efficient decision-making and it was stressed that this element must be retained. Communication with SMC was felt to be good, but may be improved further in key areas such as scoping and choice of comparators. However concern was expressed by some that patients in Scotland do not have access to innovative medicines which were available in other nations of the UK, or in other European countries. It was felt that there was a need to ensure that there was "an efficient and caring end-to-end process for patients in Scotland to be able to access the appropriate medicines, with no gaps or harmful variations between SMC, SIGN, Health Board Area Drug and Therapeutics committees, Healthcare Improvement Scotland or Regional Planning Groups, in the development of local protocols and the availability for the patient".

A view was expressed that any SMC "accepted" medicine should be available to patients in a timely manner, and there were therefore related concerns about the



process by which such medicines were considered for inclusion in individual formularies, and the speed of this process. This included the need to have a simple process for non-formulary requests of accepted medicines.

There was also a desire expressed that the Health Technology Assessment (HTA) criteria should be reviewed, and concern was expressed that the introduction in the use of modifiers had not had a major impact on the decision-making process or outcomes. In addition, it was noted that there were examples of poor implementation of SMC advice, and that a monitoring tool was necessary to address this.

### **4.3. Views of health professionals and NHS managers in Scotland**

There was widespread acknowledgement and support by this group of the quality SMC's HTA process, as well as its speed, rigour, comprehensiveness and value for money. There was also strong support for the two-stage process in which NDC's advice is considered, and sometimes modified, by SMC. The engagement between SMC and clinicians in Scotland was also cited as strength. The independence of SMC in arriving at difficult decisions was greatly appreciated and consultees noted that this independence must be retained, as it is valued so highly by health professionals in NHS Scotland. It was also observed that by offering PAS schemes, SMC was improving access to medicines for patients, whilst also achieving value for money for NHS Scotland. However some concerns were expressed about the transparency of the use of the modifiers in the HTA process, and that the process may not fully take into account the value of different stages of life and other societal influences.

Concerns were raised concerning the lack of uniformity of the IPTR process, including variation in time taken to decisions across Scotland.

### **4.4. Views of health economists**

All the health economists involved with the SMC HTA process, some SMC core staff and other independent commissioned consultants, believed that the health economic component of the present SMC process was robust and efficient, even if further improvements were possible. All the health economists consulted stated that they believed the QALY to be still the best available measure of utility, whilst recognising its weaknesses and deficiencies. They all stated that they believed that Quality Adjusted Life Year (QALY) methodology, when used appropriately, represents an effective tool to calculate cost-effectiveness of new innovative medicines in terms of off-setting the cost of the medicine against potential savings.

There was broad general support for having a policy for HTA of ultra-orphan as well as orphan medicines. It was felt to be very important that ultra-orphan products should undergo health economic appraisal, since there was an opportunity cost associated with the purchase of all medicines. The general view was that the NHS should not pay whatever premium price was required for medicines to treat patients with very rare diseases.

All felt that the use of modifiers was appropriate in certain circumstance. No-one had any criticisms of the “modifiers” identified, and felt them to be helpful in interpreting the health economic data. No potentially important new modifiers were considered to be missing in the existing list.

## 5. Findings

We were provided with all the relevant documents required to examine the HTA process in detail. The processes detailed in these documents were robust and appropriate for the purpose for which they had been developed.

**5.1.** The remit of Scottish Medicines Consortium (SMC) to provide advice to NHS Boards and their Area Drug and Therapeutics Committees (ADTCs) in Scotland concerning the status of **all** newly licensed medicines, all new formulations of medicines already licensed, and new indications for established medicines (licensed from January 2002) as close to launch as possible is unique in the UK, making it the most comprehensive of the three existing UK HTA bodies (NICE, SMC and AWMSG). When comparisons have been made between the recommendations of these HTA bodies, they have generally shown reasonably close concordance (*Cairns 2006, Webb et al 2006, Dear et al 2007, Barbieri et al 2009*). The speed and timeliness of the process is an important strength. One factor associated with variation in uptake of advice may be delay between the UK launch of a medicine and initial advice from the HTA body (*Bennie et al 2010*) so the timeliness associated with the SMC process (without sacrifice of rigour) is valuable in promoting more consistent uptake of advice.

The service which SMC provides is of great benefit to the Health Boards in ensuring that clinically effective and cost effective medicines are available in a timely fashion. The need for timely SMC advice, as early as possible after launch of a new medicine in the UK was illustrated by a review of SMC “not recommended” decisions. There was a pattern of increasing use of the new medicine the further the SMC advice was issued from the launch date (*SMC Evaluation Project Team 2008*). There is also evidence that since SMC began its work, the decisions made by ADTCs concerning local implementation of medicines have been more consistent and comprehensive and that it has reduced duplication of effort. (*Hems et al 2012*).

Its horizon scanning service provides valuable early intelligence in a timely manner so that NHS Boards plan their budgets as effectively as possible, given the uncertainties of the drug development process. The annual report “*Forward Look*” is of high quality, comprehensive and informative in this regard.

**5.2.** The membership of SMC (and its subcommittee, the New Drugs Committee [NDC]) is appropriate and representative of those groups which should be expected have an influence in the process. This includes having representatives from the Association of British Pharmaceutical Industries (ABPI) and “public partners” as lay representatives. SMC and NDC meet sufficiently frequently to provide a timely HTA service, generally the quickest of the three UK services. SMC’s committees and processes are strongly supported in their work by experienced pharmacists and health economists.

**5.3.** It adds to the efficiency of the process that NDC first conducts a review of the submissions concerning the clinical effectiveness and cost effectiveness of the medicine. This allows SMC to then also take broader matters into account, including societal issues.

**5.4.** The documentation developed to support the appraisal of full submissions is well-structured, enhancing the efficiency of the process. The information provided to manufacturers on the website in relation to full submission is clear and informative.

**5.5.** The approach of allowing abbreviated summaries allows flexibility and is proportionate. The information provided to manufacturers on the website in relation to full submission is also clear and informative.

**5.6.** The existence of an Independent Review process adds to the responsiveness and robustness of the HTA process. It is appropriate that the Independent Review panel feeds back its findings to SMC to inform any subsequent HTA decision.

**5.7.** SMC is not alone in using the cost of the medicine per Quality-Adjusted Life Year (QALYs) gained in the process used to assess the cost effectiveness of medicines. NICE and AWMSG have adopted the same measure. The US Panel on Cost-Effectiveness in Health and Medicine has also chosen the QALY when making judgements around cost-effectiveness (*Weinstein et al 2009*). The QALY takes into account the increased survival achieved by treatment and the quality of that increased life. It also allows different treatments to be compared for different conditions, thus affording a “common currency” for decision making (*National Institute of Health and Clinical Excellence 2008*).

The assumptions underlying the QALY are well described by Weinstein and colleagues (*Weinstein et al 2009*). The QALY (sometimes termed the “conventional QALY”) does not take into account issues of fairness and equity. That is why QALY estimates are only one component of the HTA process, and must be used alongside judgements of the importance of these broader societal issues, including social value judgements (*Littlejohns et al 2012*). Rawlins has stated that such issues are societal because they “relate to society rather than basic or clinical science” (*Rawlins MD 2012*) He identifies some of these social values, including whether the NHS should be prepared to pay more to extend the life of a child compared with an adult, whether treatments which prolong life at the end of life should be considered more favourably than others, whether greater weight should be given to medicines for treatments for serious conditions, and whether the NHS should be prepared to pay premium prices for treatments for very rare conditions. He points out that consideration of such social values in decision making should be determined by the values of the public who own and fund the NHS (*Rawlins MD 2005*). This issue is also discussed later in 5.7.

Other criticisms of the QALY include that it lacks sensitivity when comparing two competing but similar medicines, and when assessing the treatment of less severe health problems (*Phillips CJ 2005*). It has been noted that the QALY may be of less value in chronic diseases, where quality of life is more important than survival in calculating the QALY and the QALY assumes that the utility of a health state is not affected by the time the patient spends in that health state. Phillips has also pointed out the criticism that QALYs may attach inadequate weight to emotional and mental health problems (*Phillips CJ 2005*). However it was the universal view of the health economists consulted that QALYs remain the best available explicit measure of cost-effectiveness, and are appropriate for the comparisons made during HTA of medicines. They were also unanimous in the view that the use of the QALY represents an effective tool to calculate cost-effectiveness of innovative medicines in

terms of off-setting the cost of the medicine against potential savings. They should be considered alongside all of the other available sources of evidence by NDC and subsequently SMC.

We note that SMC does not have a formal cost effectiveness threshold because the QALY is only one (albeit important) factor informing the process of arriving at a recommendation on a specific medicine. SMC's policy is clearly stated on the website and is that "a cost per QALY of under £20,000 is generally considered acceptable value for money. For a medicine with a cost per QALY between £20,000 and £30,000 SMC might accept this if the medicine gives significant benefits over existing treatments". This policy is also closely in line with the policies of the other HTA bodies in the UK (NICE and AWMSG).

As stated earlier, decisions about medicines should not be based on evidence of relative costs and benefits alone. Littlejohns and colleagues have recently examined the websites of SMC, NICE and AWMSG to references to social value statements that have a role to play in health policy decision making (*Littlejohns et al 2012*). They found that all three HTA bodies contained statements addressing important aspects of the framework for social values applicable to health priority setting outlined previously (*Clark and Weale 2011*)

**5.8.** We agree with the principle that the use of modifiers may allow SMC to accept greater uncertainty in the health economic case or to allow acceptance of or a higher cost per QALY in some cases. The modifiers listed earlier (2.8) are all reasonable and we cannot suggest any major factors which are not present in that list.

**5.9.** In the same way, the use of additional factors in appraising orphan drugs is a valid approach, and the factors identified by SMC are all relevant, we cannot identify any major omissions in the list, which SMC clearly acknowledges in its supporting documents is not an exhaustive one.

The issue of ultra-orphan medicines deserves special mention. In 2004, NICE set up a "Citizens Council", consisting of 30 individuals (none of them health professionals) living in England and Wales, to help provide advice about social values relating to HTA decision-making. Sixteen members considered that NHS should consider paying premium prices for drugs to treat patients with very rare diseases, providing certain conditions applied. Four members considered that the NHS should pay whatever premium price was required to purchase medicines to treat patients with very rare diseases. Seven felt that the NHS should not consider paying premium prices for medicines to treat patients with very rare diseases. Instead decisions should be made using the same criteria as those used for treatments for conditions that were not considered rare. The main criteria that the Council felt should be considered in the decision were in decreasing order of importance, the degree of severity of the disease, whether the treatment would provide health gain, rather than just stabilisation of the condition, or if the disease or condition were life-threatening. (*NICE2004*).

In contrast, a recently published choice-based experiment in over 4000 UK adults using web-based surveys did not support the special funding status for treatments of rare diseases *per se* (*Linley and Hughes 2012*). The experiment did support the use

of the criteria for medicines treating severe diseases, those addressing unmet needs, those that were innovative (provided they offered substantial health benefits), and had wider societal benefits. However ultra-orphan diseases, as well as being extremely rare, are often severe, life threatening or chronically debilitating (*Hughes et al 2005*). In addition, medicines developed to treat them may also fulfil several other criteria listed above that the public consider as important in making choices.

One HTA body in the UK (AWMSG) has appraised ultra-orphan medicines since 2002 and has developed a policy to address HTA of these particular medicines, and it's most recent policy (approved in July 2012) reads as follows.

“AWMSG / NMG (New Medicines Group, analogous to the NDC in Scotland) will consider the same criteria for clinical effectiveness and cost-effectiveness of ultra-orphan medicines as those applied to other medicines. The rarity of the disease is not, in itself, a reason why an economic assessment cannot be made. However, AWMSG / NMG recognise that the evidence base will necessarily be weaker. AWMSG also recognise that the incremental cost-effectiveness ratios (ICERs) of many ultra-orphan medicines will exceed the threshold cost-effectiveness range. In such cases, AWMSG / NMG will consider evidence on the following to inform their decisions (in descending order of priority):

- The degree of severity of the disease as presently managed, in terms of quality of life and survival
- Whether the medicine can reverse, rather than stabilise the condition
- Whether the medicine may bridge a gap to a “definitive” therapy (e.g. gene therapy), and that this “definitive” therapy is currently in development
- The innovative nature of the medicine. AWMSG / NMG will consider whether the medicine:
  - I. represents a significant improvement on existing therapy (e.g. the medicine is able to treat a condition where there was previously no effective treatment) and;
  - II. whether it can plausibly generate substantial health gains over existing treatments for the individual (e.g. >1 quality-adjusted life year [QALY]) “
  - III. “Ultra-orphan medicines are appraised on a case-by-case basis, and all patients receiving approved medicines should be entered into registries for recording prospective measures of clinical outcome”.

“An advice review date may be set to ensure that additional clinical trial evidence or clinical audit data is reviewed, and this may require an additional submission” ( All Wales Medicines Strategy Group 2012).

We note the strength of views expressed by several consultees that ultra-orphan medicines should be recognised as a distinct and important and group of medicines within the orphan medicine category. Therefore we recommend below that SMC consider developing a policy for ultra-orphan medicines (see recommendations in Section 6 and also in Summary).

**5.10.** We endorse SMC's view that the use of Patient Access Schemes is a valuable approach in improving the cost-effectiveness of medicines, and therefore patient access to medicines. The criteria for accepting these schemes used by SMC are appropriate and robust. It is also appropriate that the process in Scotland is at arms-length to SMC via the separate body, the Patient Access Scheme Assessment Group (PASAG)

**5.11.** The Patient and Public Involvement Group (PAPIG) plays an important role in encouraging patient and carer involvement in SMC's work. The stated aims of the group as listed on the SMC website are comprehensive and chosen to promote public awareness of, and involvement in, the work of SMC.

The reports of the work of PAPIG published annually in the SMC annual report for the period in which they are available (between 2001-02 and 2008) are clear, informative and promote transparency.

**5.12.** Discussion with the pharmaceutical industry through the vehicle of a User Group Forum (UGF) is important in ensuring that the processes are responsive to the companies submitting their medicines for advice and SMC UGF is a useful way of ensuring relevance and usability of SMC's HTA processes.

**5.13.** In addition to its HTA role, SMC has a horizon scanning service on behalf of Health Boards. This supports financial planning, and is provided to key senior Health Board staff, after they have signed a confidentiality agreement, since some of the information is commercial-in-confidence. An annual report about the horizon scanning role, "Forward Look" has been produced each year since 2003. It gives estimates of the estimated uptake of medicines, and the estimated resultant net budget impact. This takes into account any likely replacement of existing medicines, as well as any estimated additional costs or savings associated with diagnostic testing, delivery and monitoring of the medicine. This publication is clear, informative and appreciated by those with the responsibility for financial planning, and SMC are to be commended for it.

# A way forward

## 6 – Recommendations

In our view, SMC has efficient, robust, independent and timely HTA processes. It is important that these attributes can be clearly observed by those who may be affected by the process and by the advice given to NHS Scotland by SMC. The principle of “procedural justice” adopted by NICE emphasises the importance of ensuring that the processes by which decisions are reached are transparent, and that the reasons for these decisions are explicit (*National Institute of Health and Clinical Excellence 2008*). Thus in order to further increase the transparency of the process for patients, patient groups, the public, by health professionals and by pharmaceutical manufacturers,

**Recommendation 1. SMC should meet in public so that members of the public, patients, patient group representatives, other health professionals and members of the pharmaceutical industry can attend to observe the appraisal process.**

This will allow all these groups the opportunity to hear the discussions leading up to the decision-making process. Patients and patient group representatives can observe how the patient voice provided by their submissions contributes to the discussion, and those working in the NHS or pharmaceutical industry can observe the rigour of the scrutiny being applied to the sources of evidence. One HTA body (AWMSG in Wales) has met in public since its inaugural meeting in 2002, and NICE has now conducted its appraisal meetings in public for several years. After the public documentation, any further deliberations can be held in private before the committee’s view is ascertained and announced to the audience. This may be by general assent (as is most commonly the case in SMC and NICE) or by confidential simple majority vote (as occurs at AWMSG). Because of the “commercial and in-confidence” nature of deliberations around medicines associated with a patient access scheme (PAS), part or all these particular appraisals will still need to be held in private at the present time.

**Recommendation 2. SMC should invite the manufacturer of the new medicine under consideration to give evidence at their main SMC appraisal meeting, in order to address any outstanding questions that SMC members have and highlight any outstanding issues of which they believe SMC should be aware prior to its advice being published.**

This ensures that manufacturers can correct factual errors, highlight any criticisms of the process, and clarify any outstanding issues for committee members before they make a judgement. It thus also allows the manufacturer to be assured that no relevant issues have been overlooked, and that the subsequent committee decision is made based on a sound and complete evidence-base. It also allows them to give assurances to the Committee that they believe the process to have been fair and transparent.



It is disappointing when manufacturers decide not to engage with a HTA process. Non-engagement can result in uncertainty within the NHS regarding the clinical effectiveness and cost effectiveness of the medicine for that particular indication. While some of these non-engagements may be for indications for which alternative treatments are already available, some may be for innovative medicines, which may have potentially significant benefits for patients.

It is essential to ensure that the medicines appraisal process continues to remain comprehensive, and provides timely advice to NHS Scotland on all relevant new medicines. To address the challenge associated with non-engagement of manufacturers with the SMC process (this was 30% of all new medicines in 2012) and guided by the views of Health Boards, ADTCs and clinicians:

**Recommendation 3. SMC should be able to appraise any new medicines which the NHS in Scotland considers potentially of major importance to patient care, but which have not been submitted to SMC by the manufacturer within 3 months of launch. If necessary this appraisal may be conducted using such data as is already available in the public domain.**

This helps to reinforce the centrality of the patient in the HTA process, as well as the importance of the views of clinicians, who are striving to do the best they can for their patients. While a “not recommended” notice may be issued when non-engagement occurs, clinicians may still remain uncertain of the clinical effectiveness of the medicine and its potential place in treatment. They can therefore benefit from access to a rigorous and independent appraisal of the evidence drawn together from available sources already in the public domain. Information on these medicines can be obtained from SMC’s well-developed horizon scanning process, as well as by regular dialogue with the appropriate clinicians and patient groups in Scotland and with the ADTCs.

In order to further improve the responsiveness of the HTA process, it is vital that there is close dialogue between the HTA body and the manufacturer (e.g. in scoping, choice of comparators etc.) from the beginning of the process. To further facilitate this interaction,

**Recommendation 4. SMC should be able to have a temporary pause in the appraisal process at any stage in order to permit further dialogue with manufacturers on issues that would be likely to be central to the subsequent decision-making process.**

While it is possible to conduct a reappraisal, this process can be expensive and result in duplication of preparation of evidence and wasted committee time, resulting in unnecessary expense for all parties. It may also result in the issuing of several notes of advice for the same medicine in a particular indication. Using this option when SMC considered it appropriate should help to reduce the resubmission rate, and while the advice might be slightly delayed by such consultation, it would improve efficiency, clarity of recommendation, and ensure that the SMC process was even more responsive than is the case already.

In order to further address the specific challenges associated with “ultra-orphan” medicines (those medicines licensed for the treatment of diseases with a UK prevalence of less than 1 in 50,000)

**Recommendation 5. SMC should develop a policy specifically relating to ultra-orphan medicines to guide the process of consideration of all available evidence relevant to its advice on these medicines.**

Elsewhere, methods to assess the views of the public such as a “Citizen’s Councils” or “Citizen’s Jury” have been successfully used to explore specific societal issues in relation to the availability of new medicines. To further improve SMC’s engagement with patients and the general public,

**Recommendation 6. SMC, with the appropriate resource, and in partnership with other relevant bodies in Scotland, should be encouraged to set up an engagement process such as a “Citizen’s Council” or “Citizen’s Jury” to explore views around specific societal issues of importance to the people of Scotland in relation to the availability of new medicines, and the impact of these views on the existing processes for ensuring access to medicines.**

This work can be led by the public partners and the Patient and Public Involvement Group (PAPIG), who are ideally placed to help to build on SMC’s existing strong commitment to further improving transparency of the HTA process.

**Recommendation 7. SMC should explore other innovative approaches to increasing patient and public awareness of its role in ensuring timely access to clinically effective and cost-effective medicines in Scotland. Consideration should also be given to expansion of its role to support other aspects of safe, effective and cost-effective prescribing. SMC should produce a publicly available annual report of progress in this regard detailing its important contributions to this objective.**

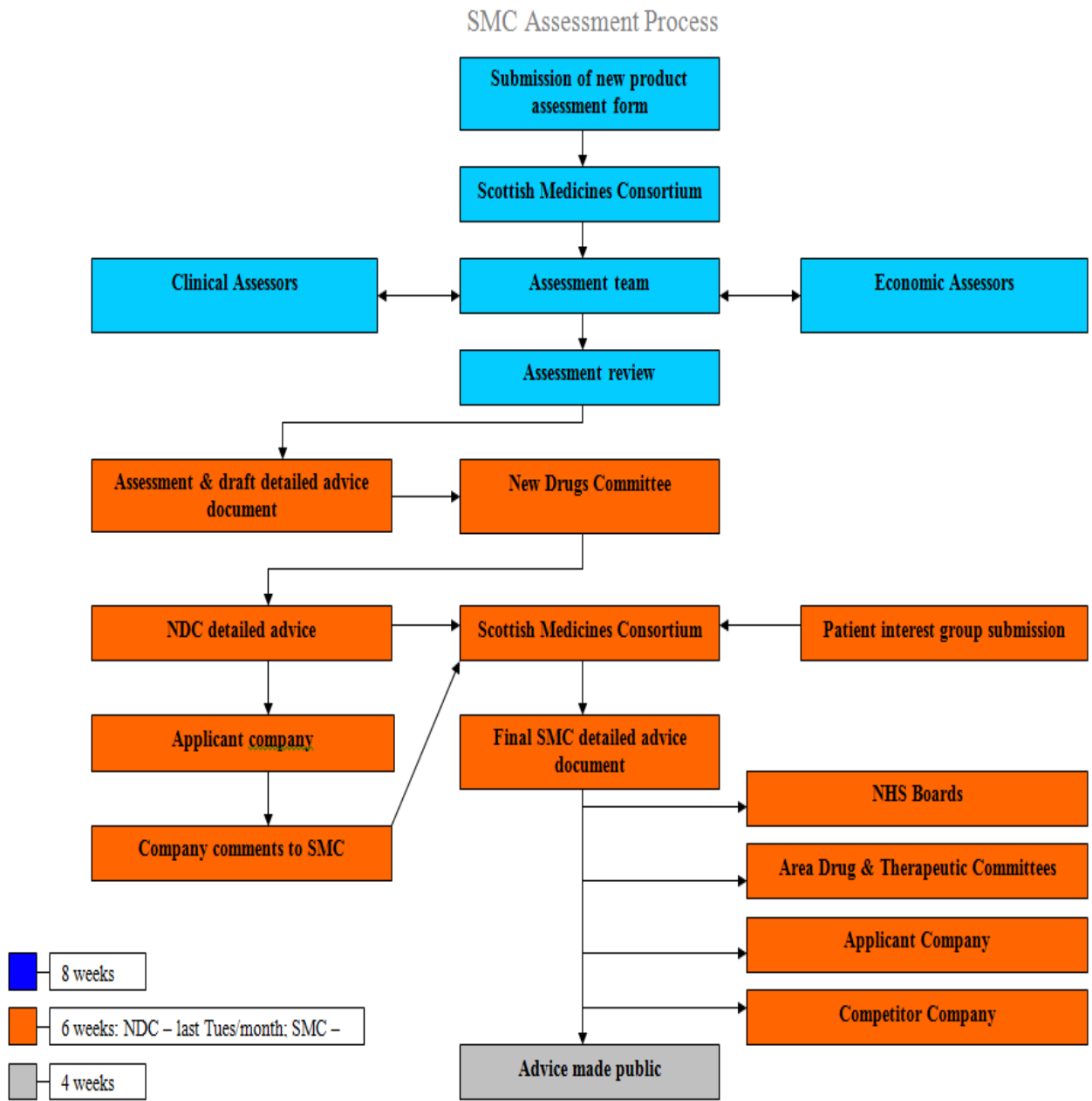
Value Based Pricing (VBP) is intended to ensure that the prices of new individual branded medicines reflect their ‘clinical and therapeutic value to patients and the broader NHS. It is designed to link the pricing of a medicine to the benefit it has been shown to deliver, so that access to new medicines can be further improved. The Office of Fair Trading (OFT) noted that ‘in the interests of patients, it is vital that NHS resources be used cost effectively. Since their creation, NICE, SMC and AWMSG have made a significant contribution to achieving this aim’. The OFT report on The Pharmaceutical Price Regulation Scheme: An OFT market study also stated that “NICE, SMC and AWMSG are natural candidates for conducting the cost effectiveness analysis required to implement value-based pricing” (*OFT2007b*). These skills within SMC could be harnessed to support the development and delivery of VBP in the future.

**Recommendation 8. NHS Scotland should explore ways in which the expertise available within SMC could be used to support the process of Value Based Pricing (VBP).**

When a medicine is not recommended, it is important that The Individual Patient Treatment Request (IPTR) process possess the same qualities (transparency, timeliness, relevance, in-depth robustness, and usability) as an ideal HTA process. For these reasons,

**Recommendation 9. A register of IPTR decisions in all Health Boards, suitably anonymised to protect patient confidentiality should be kept, and supporting information related to IPTRs shared between Health Boards.**

**Recommendation 10. There should be regular sharing of experiences between the IPTR panels and members of IPTR panel members across Scotland should meet at least annually for feedback and training.**



**Figure 1. The process used by SMC for Health Technology Appraisal**

## References

All Wales Medicines Strategy Group (2012). AWMSG policy relating to Ultra-orphan Medicines

<http://www.wales.nhs.uk/sites3/docmetadata.cfm?orgid=371&id=195381>

Accessed 28/02/2013

Anon. Working with SMC – A Guide for Manufacturers (2012)

[www.scottishmedicines.org.uk/.../Working with SMC FINAL July 2012](http://www.scottishmedicines.org.uk/.../Working_with_SMC_FINAL_July_2012)

Accessed 21/02/2013

Barbieri M, Hawkins N, Sculpher M. Who does the numbers? The role of third-party technology assessment to inform health systems' decision-making about the funding of health technologies.

Value in Health 2009;12:193e201.

Bending M, Hutton J, McGrath C. A comparison of pharmaceutical reimbursement agencies' processes and methods in France and Scotland.

Int J Technol Assess Health Care. 2012; 28: 187-94

Bennie M, Dear J, Hems S, Black C, Mclver L, Webb DJ. An investigation into the effect of advice from the Scottish Medicines Consortium on the use of medicines in Scotland's Health Service.

Br J Clin Pharmacol. 2011; 71: 283-8.

Cairns J. Providing guidance to the NHS: The Scottish Medicines Consortium and the National Institute for Clinical Excellence compared.

Health Policy. 2006; 76: 134-43. Epub 2005 Jun 27.

Clark, S. and Weale, Social values in health priority setting.

Journal of Health Management and Organisation 2012; 26: 293-316.

Dear J, O'Dowd C, Timoney A, Paterson KR, Walker A, Webb DJ. Scottish Medicines Consortium: an overview of rapid new drug assessment in Scotland.

Scott Med J. 2007; 52: 20-6

Ford JA, Waugh N, Sharma P, Sculpher M, Walker A. NICE guidance: a comparative study of the introduction of the single technology appraisal process and comparison with guidance from Scottish Medicines Consortium.

BMJ Open. 2012; 2: e000671. Print 2012.

Healthcare Policy and Strategy Directorate: Quality Division. Introduction and availability of newly licensed medicines in the NHS in Scotland.

[http://www.sehd.scot.nhs.uk/mels/CEL2010\\_17.pdf](http://www.sehd.scot.nhs.uk/mels/CEL2010_17.pdf)

Accessed 15/03/2013

Hems S, Black C, Mclver L, Bennie M. Equity and efficiency: striving for a solution for the introduction of new medicines.

Scott Med J. 2012; 57: 191-5. Doi: 10.1258/smj.2012.012115.

Hughes D, Tunnage B and Yeo ST. Drugs for exceptionally rare diseases: do they deserve special status for funding?

Q J Med 2005; 98: 829–836

Kohli HS. Scottish Medicines Consortium.

National Med J India. 2005; 18: 162.

Lexchin J, Mintzes B. Medicine reimbursement recommendations in Canada, Australia, and Scotland.

Am J Manag Care. 2008; 14: 581-8.

Linley WG & Hughes DA. Societal views on NICE, Cancer Drugs Fund and value-based pricing criteria for prioritising medicines: a cross sectional survey of 4118 adults in Great Britain.

Health Econ. 2012 Sep 7. doi: 10.1002/hec.2872. [Epub ahead of print]

Littlejohns P, Yeung K, Clark S, Weale A. A proposal for a new social values research program and policy network.

J Health Organ Manag. 2012; 26: 407-21.

National Institute for Clinical Excellence. NICE Citizens Council Report. Ultra-Orphan Drugs. London, NICE, 2004.

[www.nice.org.uk/niceMedia/pdf/Citizens\\_Council\\_Ultraorphan.pdf](http://www.nice.org.uk/niceMedia/pdf/Citizens_Council_Ultraorphan.pdf)

Accessed 28/02/2013

National Institute for Health and Clinical Excellence. Social Value Judgements: Principles for the development of NICE guidance. Second edition. 2008.

<http://www.nice.org.uk/media/C18/30/SVJ2PUBLICATION2008.pdf>

Accessed 14/03/2013

NHS Greater Glasgow and Clyde. Policies related to the management of medicines. Section 5. Non-formulary processes

[www.medlaw.eu/lhb\\_scotland/5.2 iptr policy - 1207.pdf](http://www.medlaw.eu/lhb_scotland/5.2_iptr_policy_-_1207.pdf)

Accessed 15/03/2013

Office of Fair Trading 2007. Annexe B. *Review of NICE, SMC and AWMSG*.

February 2007 (Crown copyright)

[http://www.offt.gov.uk/shared\\_offt/reports/comp\\_policy/oft885b.pdf](http://www.offt.gov.uk/shared_offt/reports/comp_policy/oft885b.pdf)

Office of Fair Trading 2007b. The Pharmaceutical Price Regulation Scheme: An OFT market study:[http://www.offt.gov.uk/shared\\_offt/reports/comp\\_policy](http://www.offt.gov.uk/shared_offt/reports/comp_policy)

Phillips CJ. Health Economics, an introduction for health professionals. Blackwell Publishing, BMJ Books 2005. ISBN 0-7279-1849-4.

Rawlins MD. Pharmacopolitics and deliberative democracy. Clin Med 2005; 5: 471-5

Rawlins MD. Crossing the fourth hurdle. BJ clin Pharmac 2012; 73(6):855-60. doi: 10.1111/j.1365-2125.2012.04263.x

Scottish Government. Implementing CEL17 (2010): Introduction and availability of newly licensed medicines in the NHS in Scotland – good practice guidance for NHS Board Management of individual patient treatment requests (IPTRs). SGHD/CMO(2011)3

Scottish Government: Guidance to further strengthen the safe and effective use of new medicines across the NHS in Scotland. SGHD/CMO(2012)1

SMC Evaluation Project Team. SMC 'not recommended' advice - an investigation of medicines use across NHS Scotland. Executive Summary and Full Report. Scottish Medicines Consortium, September 2008.

<http://www.scottishmedicines.org.uk/smc/files/Not%20Recommended.pdf>

*Accessed 28/02/2013*

Taylor R & Taylor R. What is health technology assessment?

[http://www.medicine.ox.ac.uk/bandolier/painres/download/whatis/What\\_is\\_health\\_tech.pdf](http://www.medicine.ox.ac.uk/bandolier/painres/download/whatis/What_is_health_tech.pdf)

*Accessed 14/03/2013*

Vegter S, Rozenbaum MH, Postema R, Tolley K, Postma M. J Review of regulatory recommendations for orphan drug submissions in the Netherlands and Scotland: focus on the underlying pharmacoeconomic evaluations.

*Clin Ther.* 2010; 32: 1651-61.

Webb DJ, Paterson KR, Timoney A, Walker A. Scotland v England deal on prescribed drugs: Scottish Medicines Consortium responds. *BMJ.* 2006;333: 1073; author reply 1073.

Weinstein MC, Torrance G, McGuire A. QALYs: The Basics. *Value Health.* 2010; 13: 1065.

## Glossary of terms and abbreviations

**ABPI:** see Association of the British Pharmaceutical Industry

**ADTC:** see Area Drug and Therapeutics Committee

**All Wales Medicines Strategy Group (AWMSG):** established in 2002 to bring together NHS clinicians, pharmacists, healthcare professionals, academics, health economists, industry representatives and patient advocates to provide advice on strategic medicines management and prescribing to the Minister for Health & Social Services.

**All Wales Prescribing Advisory Group (AWPAG):** formed in October 2003 to advise AWMSG on strategic developments in primary and secondary care. It assists with monitoring prescribing, advising on prescriber training, and developing prescribing indicators and audits for a national incentive scheme.

**Area Drug and Therapeutics Committees (ADTCs):** committees within each of the 14 Scottish Health Boards to support the safe and effective user of medicines by prescribers and the local population

**Association of the British Pharmaceutical Industry (ABPI):** the trade association for more than 70 companies in the UK producing prescription medicines. Its member companies research, develop, manufacture and supply more than 80 per cent of the medicines prescribed through the National Health Service (NHS).

**AWMSG:** see All Wales Medicines Strategy Group

**Citizen's Council:** A panel of 30 members of the public, which provides NICE with a public perspective on overarching moral and ethical issues that NICE must take into account when it produces guidance.

**CUA:** see Cost Utility Analysis

**Cost Utility Analysis (CUA):** A method of cost-effectiveness analysis that uses the Quality adjusted life year (QALYs) as a measure.

**EMA:** see European Medicines Evaluation Agency:

**European Medicines Evaluation Agency (EMA):**

<http://www.emea.europa.eu/htms/aboutus/organigramme.htm> a decentralized body of the European Union with headquarters in London. Its main responsibility is the protection and promotion of public and animal health, through the evaluation and supervision of medicines for human and veterinary use.

**Healthcare Improvement Scotland (HIS):** A Scottish Health body which supports healthcare providers in Scotland to deliver high quality, evidence-based, safe, effective and person-centred care; and to scrutinise those services to provide public assurance about the quality and safety of that care.

**Health Technology Appraisal/ Assessment (HTA):** “a multidisciplinary activity that systematically examines the safety, clinical efficacy and effectiveness, cost, cost-effectiveness, organisational implications, social consequences, legal and ethical considerations of the application of a health technology – usually a drug, medical device or clinical/surgical procedure”



(Taylor and Taylor 2009).

**HTA: see Health Technology Appraisal/ Assessment**

**HIS: see Healthcare Improvement Scotland**

**Horizon-Scanning:** the systematic examination of potential future developments (e.g. in treatments) which are at the margins of current thinking and planning.

**ICER: see Incremental Cost effectiveness ratio**

**Incremental Cost effectiveness ratio (ICER):** The difference in costs divided by the difference in benefits (Phillips 2005).

**Individual Patient Treatment Request (IPTR):** A process by which a patient may receive a medicine within its licensed indications when, SMC or NHS HIS has yet to issue advice on the medicine, SMC or NHS HIS has issued “not recommended” advice for the medicine (including medicines not recommended by SMC due to company non-submission) or the request relates to the use of the medicine outwith an SMC restriction (NHS Greater Glasgow and Clyde).

**IPTR: see Individual Patient Treatment Request**

**Medicines Appraisal:** The structured evaluation of the properties and effects of a medicine, ideally with consideration of its clinical effectiveness and cost effectiveness when used for the specified indication (see Health Technology Appraisal/Assessment).

**National Institute for Health and Clinical Excellence (NICE):** established in 1998, it is an independent organization responsible for providing national guidance on the promotion of good health and the prevention and treatment of ill health.

**New Drugs Committee (NDC):** formed in May 2002 to enable SMC to manage the broadened appraisal process. This group meets month to consider the evidence on new medicines, and to provide preliminary recommendations to SMC on the introduction of these medicines in Scotland.

**NICE: see National Institute for Health and Clinical Excellence**

**Office of Fair Trading (OFT):** the UK's consumer and competition authority, which aims to make markets work well for consumers.

**Off-label:** Use of a medicine outside the terms of its official labelling.

**Off-Licence:** See off-label

**OFT: see Office of Fair Trading**

**Opportunity cost:** the cost of a unit of a resource is the benefit that would be derived from using it in its best alternative use.

**Orphan Medicine:** One licensed for treating or preventing life-threatening rare diseases affecting fewer than 5 in 10,000 people in the European Union”. This equates to around 2, 500 prevalent cases in Scotland.

**PAPIG: see Patient and Public Involvement Group**

**PASAG: see Patient Access Scheme Assessment Group**

**Patient Access Scheme (PAS):** Methods by which manufacturers of medicines can offer means to enable patients to gain access to high cost medicines.

**Patient Access Scheme Assessment Group (PASAG):** The group which assesses proposed Patient Access (PAS) Schemes for acceptability in NHS Scotland against standard objective criteria.

**Patient and Public Involvement Group (PAPIG):** a subgroup of the Scottish Medicines Consortium (SMC) , which makes recommendations to SMC on the development of public involvement opportunities and ensures that the patient/carer perspective is reflected in the deliberations of SMC

**Pharmaceutical Price Regulation Scheme (PPRS):** A voluntary agreement between the UK Government and the Association of the British Pharmaceutical Industry (ABPI) which allows pharmaceutical companies to set their own prices for branded prescription medicines, but with constraints placed upon overall profit.

**PPRS: see Pharmaceutical Price Regulation Scheme**

**QALY: see Quality adjusted life Year**

**Quality adjusted life Year (QALY)** A measurement that takes into account the extent to which a treatment both prolongs and improves the quality of a patient's life. A QALY is calculated mathematically by multiplying the number of additional years of life achieved by a treatment by a measure of the quality of life. The cost-effectiveness of treatments can be compared by evaluating the cost of the treatment per QALY gained (Cost per QALY).

**Scottish Medicines Consortium (SMC):** Body formed in 2002 to provide advice to NHS Boards and their Area Drug and Therapeutics Committees (ADTCs) across Scotland about the status of all newly licensed medicines, all new formulations of existing medicines and new indications for established products.

**Single Technology Appraisal:** A single technology appraisal covers a single technology (e.g. a medicine) for a single indication.

**SMC: see Scottish Medicines Consortium**

**SMC UGF: see User Group Forum (SMC UGF):**

**Ultra Orphan Medicine:** Medicines that are licensed for the treatment of diseases with a UK prevalence of less than 1 in 50,000. This equates to around 100 prevalent cases in Scotland.

**User Group Forum (SMC UGF):** A subgroup of SMC which identifies and address and resolve issues related to the HTA processes of SMC

## **Consultees**

Professor Bill Scott, Chief Pharmaceutical Officer, Scottish Government Health and Social Care Directorates

Dr Sara Davies, Public Health Consultant, Scottish Government Health and Social Care Directorates

Mr Colin Brown, Deputy Director, Scottish Government Health and Social Care Directorates

Mrs Veronica Moffat, New Medicines Policy Lead, Scottish Government

Dr Frances Macdonald, Chairman, SMC User Group Forum

Dr Iain Wallace, Medical Director, NHS Forth Valley (by teleconference)

Dr Barclay Goudie, General Practitioner, NHS Tayside (by teleconference)

Professor Colin Suckling, Chairman, SMC Patient and Public Involvement Group

Mr Robert Calderwood, Chief Executive Officer, NHS Greater Glasgow and Clyde

Dr Brian Robson, Executive Clinical Director, Healthcare Improvement Scotland

Mrs Laura McIver, Chief Pharmacist, Healthcare Improvement Scotland

Ms Susan Went, Director for Evidence and Improvement, Healthcare Improvement Scotland

Ms Christine Gilmour, Acting Chairman Area Drug and Therapeutic Committee  
NHS Lanarkshire

Dr Simon Hurding, GP Advisor to Medicines Management Team, NHS Lothian

Ms Jane Pearson, Formulary Pharmacist, NHS Lothian

## **SMC staff and consultants**

Prof Angela Timoney, Chairman SMC

Dr Jonathan Fox, Vice Chairman, SMC, Chairman, New Drugs Committee

Ms Anne Lee, Chief Pharmaceutical Adviser, SMC

Ms Rosie Murray, SMC Manager

Ms Ailsa Brown, SMC Lead Health Economist

Mrs Corinne Booth, SMC Senior Health Economist

Mr Keith Tolley, Independent Health Economist

Dr Andrew Walker, University of Glasgow

Ms Joyce Craig, Independent Health Economist

Ms Lisa Wilson, Health Economist, Healthcare Improvement Scotland

## **Other consultees**

Prof Dyfrig Hughes, Health Economist, Bangor University

Prof Ceri Phillips, Health Economist, Swansea University

Dr John Watkins, Consultant in Public Health Medicine, Public Health Wales

## **Written submissions to the Review**

Association of British Pharmaceutical Industries (ABPI) Scotland

Cystic Fibrosis Trust

Prostate Cancer UK

Health and Social Care Alliance, Scotland

Ken Macintosh MSP

Merck Serono

PNH Scotland

Prostate Cancer UK

# NEW MEDICINES REVIEW

THE ROLE AND REMIT OF NHS BOARD AREA  
DRUG AND THERAPEUTIC COMMITTEES

AND

INDIVIDUAL PATIENT TREATMENT REQUEST  
ARRANGEMENTS

APRIL 2013

## REMIT OF REVIEW OF ADTCs and IPTRs

In November 2012, I agreed to chair a short life working group to refresh the role and remit of the Area Drug and Therapeutic Committees (ADTC); in November 2012 the Cabinet Secretary for Health and Wellbeing announced a more comprehensive New Medicines Review. I agreed to extend my examination of the function and role of ADTC and to examine the extant Individual Patient Treatment Requests (IPTR) arrangements and report to the Chief Pharmaceutical Officer. The published remit is attached at **Appendix 1**.

In December 2012, the Health and Sports Committee of the Scottish Parliament considered petitions submitted on behalf of patients with very rare diseases who were unable to access medicines in Scotland. These very rare conditions are termed “ultra-orphan<sup>1</sup>” conditions and are widely accepted as those occurring in less than 1000 people in the UK (equating to less than 100 people in Scotland). Scottish Medicines Consortium (SMC) “not recommended” advice for these conditions may be made either on the basis of a “non-submission” or because the clinical and cost-effectiveness has not been demonstrated. In January 2013, I provided interim advice to the Cabinet Secretary for Health and Wellbeing to consider the availability of a new Orphan Drugs Fund. The Cabinet Secretary announced that this fund would be available from March 2013, and I have considered proposals for how this would operate alongside the existing arrangements.

## CONTEXT

The introduction of new medicines to NHS Scotland is important for patients, the NHS and society. In October 2012, Medicines and Healthcare Regulatory Agency (MHRA) confirmed there were over 12,000 market authorisations for all forms and strengths of Prescription Only Medicines (POM) containing 1500 different active substances available in the UK for regular use and the SMC consider about 60-80 of these each year. The Charles River Associates report<sup>1</sup> demonstrated that Scotland is not significantly different from other EU countries with regard to access to **novel**<sup>2</sup> medicines. The proportion of positive recommendations was higher than Canada, the rest of the UK or Sweden. This report found that the SMC demonstrated an increased speed in appraisal for novel medicines when compared to other submissions and was the only country included in the study where this was the case.

The arrangements for the introduction of new medicines into NHS Scotland were detailed in CEL 17 (2010) and expanded further in SGHD/CMO (2012) 1. The SMC makes decisions on which licensed medicines are ‘accepted for use’, and which are

---

<sup>1</sup>Although there is no formal, or internationally agreed, definition of an ultra-orphan disease, the National Institute for Health and Clinical Excellence (NICE) uses the term for conditions occurring in less than 1000 people in the UK (equating to less than 100 people in Scotland).

<sup>2</sup> Novel medicines in this context mean a new chemical entity where the mode of action is different to that of existing medicines.

not, in NHS Scotland. SMC decisions are advisory but NHS Boards are expected to take full account of SMC advice and ADTCs are expected to make local decisions about SMC “accepted” medicines quickly.

However, only about one third of SMC advice is related to **novel** medicines. The majority are new formulations of **existing** medicines or new chemical entities within a class of **existing** medicines and may offer no or limited evidence of increased clinical benefit to patients, and often at increased costs. SMC may accept these for use in Scotland because the submitted evidence shows clinical and cost effectiveness and acceptable short term safety. SMC does examine comparative clinical effectiveness and cost-effectiveness with medicines in use already as part of their judgement, and comparative data may be submitted; however it is the task of local ADTC to make the final judgement to introduce SMC “accepted” medicines based on local consideration of clinical benefits within the local context and the preferences of local prescribers. Access should not be judged solely on the number or value of all **new** medicines made available but on access to **effective** medicines and treatment, which will include novel medicines and many that are in use already.

One of the important roles of the ADTC (who are all represented at the SMC) is to create local formularies from all available prescription only drugs; these local decisions are agreed by hospital consultants and GPs. A formulary represents a choice of medicines, often with a preferred first choice, followed by a number of additional choices to cover the needs and clinical circumstances, of all local patients including where patients may be intolerant of a particular medicine. Formularies have general sections for use by all prescribers for commonly used medicines, and specialist sections for use by only approved specialists in that field. The formulary represents a balance of new medicines where there are improvements in effectiveness, the effectiveness of existing medicines, convenience for patients, local knowledge and familiarity with actions and side effects, and costs. Familiarity with expected and unexpected effects of medicines is critical to patient safety; the well-publicised withdrawal of a COX inhibitor anti-inflammatory medicine because excess heart attacks emerged after trials and licensing and was informed by prescribers encountering unexpected cases of heart disease.

Medicines’ spending accounts for 15% of NHS budgets or about £1.4 billion each year and has risen steadily over the past 5 years. NHS Boards are accountable for managing all resources allocated to them and achieving financial balance each year. Medicines budgets have been rising steadily as NHS Boards recognise the need for new and costly medicines and an ageing population requiring treatment. Prescribing costs are funded from NHS Board baseline budgets provided by the Scottish Government and it is for NHS Boards to ensure that they effectively monitor and review the costs of prescribing within their overall funding envelope. GPs are free to prescribe any of the 12,000 licensed medicines and it is a key feature of local ADTC that GPs are engaged to prescribe responsibly. A formulary therefore

- ✓ supports consistent good quality prescribing
- ✓ helps patients and doctors to be familiar with the effectiveness and side effects of medicines
- ✓ enables effective teaching
- ✓ supports budget planning

- ✓ enables the introduction of novel medicines in a context of knowledge and practice about local patients, existing medicines and treatments, and the assessment of new medicines in comparison to those already in use
- ✓ promotes local ownership by GPs and hospital doctors of prescribing
- ✓ promotes local choice of medicines for patients and prescribers.

Formularies are advisory; prescribers may prescribe legally any licensed medicine they believe to be in the best interests of a patient.

NHS Clinicians may prescribe SMC “accepted” medicines which are not on the NHS Board formulary through a straightforward “Non Formulary” request. My discussions with stakeholders did not indicate any difficulties in accessing medicines in these circumstances.

## REVIEW OF ADTCs – SUMMARY OF RECOMMENDATIONS

The overall picture is one of sound arrangements for the managed introduction of new medicines within the NHS in Scotland that can be improved further. Outside the NHS there is uncertainty, lack of knowledge, and limited access to information about ADTC processes from the NHS; inside the NHS there are sound guidance and examples of excellent arrangements that are marred by inconsistency, failure to fully follow guidance and a mixed picture of availability of transparent and easily accessed information for patients and the public. My recommendations are aimed at improving these aspects.

### RECOMMENDATION 1

Board ADTC should publish their local response to the SMC published advice within 30 days of the SMC advice, on the Board website and in a manner which is accessed easily by the public and patients (as required by CMO 1 2012). The response need not be definitive if further work is required but should indicate clearly the Board’s intentions; the final arrangements should be published within 90 days. Members of the public involved in the work of the ADTC (drawn from the members of the Board Patient and Public Forum (PPF) can assist with describing the processes in a way that is “user-friendly” for the general public, and act as a link with the wider PPF.

### RECOMMENDATION 2

Board ADTC should publish their decisions and the reasons for their decisions in respect of SMC advice to be compliant with CMO (2012)<sup>1</sup>. These reasons should include the consequences for the local formulary, even if, in the case of novel medicines, this requires further deliberation and planning. Patients and the public should be signposted from the front page of the Board website to a link which will provide information about recent SMC decisions and subsequent formulary decisions



and the overall formulary should be published alongside this information and updated as required.

### **RECOMMENDATION 3**

Board ADTC should demonstrate the engagement of their PPF in the work of the ADTC. For preference, Board ADTC should have at least one member drawn from the PPF or demonstrate the connection between the PPF and the work of the ADTC

### **RECOMMENDATION 4**

NHS Scotland should consider a national meeting of all relevant specialists, organised by Healthcare Improvement Scotland (HIS) , to agree a national implementation plan for some new medicines accepted by the SMC that meet agreed criteria. These criteria may include the introduction of novel, first in class medicines where there is considerable uncertainty of its place in therapy. The plan will apply to all patients covered by the SMC “accepted” advice and to all Boards to support equity of access. Further, HIS should continue to audit access to new medicines and compliance with CEL 17 (2012) and SGHD/CMO (2012) 1.

### **RECOMMENDATION 5**

NHS Scotland should retain the existing ADTC to maintain alignment of patient and GP interests, safe prescribing and enable Boards to manage their costs. Regional clinical networks could have a role in agreeing equitable access to new medicines in relation to their populations.

## **REVIEW OF IPTRs – SUMMARY OF RECOMMENDATIONS**

### **RECOMMENDATION 6**

All Boards should adopt the same IPTR paperwork and process, based on the examples from Greater Glasgow and Clyde, Lothian or Grampian. The application should contrast the clinical criteria appraised by the SMC where “not recommended” advice has been published with the patient’s disease and personal clinical characteristics so that the reasons for the IPTR are more easily assessed, and can be audited.

### **RECOMMENDATION 7**

The IPTR arrangements in Boards should be audited by HIS to assess compliance with guidance and its consistency of application, and to publish the results.

### **RECOMMENDATION 8**

Clinicians should be provided with basic training and guidance in the IPTR process locally. Clinicians who are uncertain or inexperienced should be able to access specialist advice and support (see recommendation 10).

### **RECOMMENDATION 9**

Boards should consider whether IPTR panels should include a member of the public drawn from the Board's patient and public forum. Member(s) will require training and support.

### **RECOMMENDATION 10**

All doctors considering an IPTR must be able to access consistent, knowledgeable support for their patients. National Services Division (NSD) should establish and maintain a register of approved specialists to support IPTR. One specialist may be sufficient for orphan and ultra-orphan diseases, but more than one specialist may need to be available for more common diseases, or variants, and on a regional basis. The model of the cancer networks is an example.

### **RECOMMENDATION 11**

The Scottish Government and Boards should produce clear and concise documentation, available on national and local websites, that explains the roles of ADTC and IPTR, how the public and patients can be involved, and provide links to the reports recommended above and for ADTC.

### **RECOMMENDATION 12**

The RCMF should focus on access to medicines for ultra-orphan diseases. Access should be supported where the SMC has published 'not recommended' advice after a full submission of the medicine, **and** after a successful IPTR or GPTR has been agreed.

## REVIEW OF AREA DRUGS AND THERAPEUTICS COMMITTEES

The short life working group had agreed new guidance on the function and roles of ADTC, attached as **Appendix 2**. However, during the course of my examination of IPTR, reading the reports of the recent Health and Sports Committee public sessions and the evidence submitted, and listening to the views of patient and public representatives, a number of further concerns about ADTC have been articulated. Healthcare Improvement Scotland (HIS) have conducted an audit of access to new medicines in NHS Boards which is attached at **Appendix 3** and which provides some helpful factual information relevant to my report.

### HOW WELL ARE ADTC WORKING?

SMC is admired widely for the speed of its appraisal process, but the availability of accepted medicines to local populations is viewed by patients, the industry and members of the public as very slow.

However –the Medicines Access audit conducted by Healthcare Improvement Scotland for 23 SMC “accepted” medicine decisions published between April 2012 and September 2012, indicated that the average uptake of these on to Board formularies was 74%. This suggests that the majority of SMC “accepted” medicines are made available across NHSScotland.

ADTC decisions appear to some public representatives and those that gave oral evidence to the Health and Sports Committee to be less than transparent, arbitrary, inconsistent and slow to members of the public, including industry, or patients seeking local information. This is in stark contrast to the advice of the SMC which is made publicly available together with the reasons for that advice.

CMO (2012)<sup>1</sup> detailed 6 categories for Boards to record decision making, ranging from **included on formulary** through **pending protocol** or **awaiting response from prescribers** to **not included**. This means that Board ADTC can make decisions on new accepted for use medicines within 2 weeks of the SMC in one of these categories for each new medicine. Board ADTC decisions could be available within 30 days of the SMC recommendation, and be updated as the decision is progressed, with a final decision by 90 days. The changing status of the decision could be updated as required and the transparency and speed of the process should be available to the public and patients.

The HIS Medicines Access Audit found that there is a mixed picture of availability of easily accessed information for patients and the public and identified 7 Boards which provided easy access to ADTC decisions and access to their formularies, 3 provided more limited access and 4 had no information about ADTC or formulary. An example of internal good practice is NHS Lothian where prescribers are provided with a summary of SMC decisions and the ADTC advice for the formulary on the

Board intranet and a separate formulary website – although this is not easily accessible to the public.

## **RECOMMENDATION 1**

Board ADTC should publish their local response to the SMC published advice within 30 days of the SMC advice, on the Board website and in a manner which is accessed easily by the public and patients (as required by CMO 1 2012). The response need not be definitive if further work is required but should indicate clearly the Board's intentions; the final arrangements should be published within 90 days. Members of the public involved in the work of the ADTC (drawn from the members of the Board Patient and Public Forum (PPF)) can assist with describing the processes in a way that is "user-friendly" for the general public, and act as a link with the wider PPF.

ADTCs appear to members of the public to reach different decisions in different Boards. From the public perspective, the ADTCs responses to SMC new drug advice appear to be significantly different; there appears to be little evidence available that links the published SMC advice to the evolution or development of local formularies. It is difficult to determine whether SMC advice has been considered nor are the reasons for ADTC decisions evident. CMO (2012)<sup>1</sup> required the SMC to indicate to Boards when a new medicine represents a therapeutic advance over comparative medicines which should help with decision making locally, and that is starting to happen. For the 23 medicines described in the HIS report, the average uptake on to Board formularies was 74%. The reasons for not including the remaining 26% needs to be explored further to determine whether it reflects different local service provision or inconsistent use of the decisions categories set out in SGHD/CMO (2012) 1 (or both).

## **RECOMMENDATION 2**

Board ADTC should publish their decisions and the reasons for their decisions in respect of SMC advice to be compliant with CMO (2012)<sup>1</sup>. These reasons should include the consequences for the local formulary, even if, in the case of novel medicines, this requires further deliberation and planning. Patients and the public should be signposted from the front page of the Board website to a link which will provide information about recent SMC decisions and subsequent formulary decisions and the overall formulary should be published alongside this information and updated as required.

Patient and public representatives told me that Board ADTC are inconsistent with respect to public involvement in their work. A few ADTC have one or two members drawn from the Board PPF. NHS Forth Valley is a good example where the PPF member contributes to the work of the ADTC and reports to the PPF. Others use public representatives on ADTC sub-committees. The advantages of involving public members include assistance with understanding the public reaction to SMC advice,

support with communication of decisions to patients and the public, and consideration of the public concerns with formulary development and the introduction of new medicines.

### **RECOMMENDATION 3**

Board ADTC should demonstrate the engagement of their PPF in the work of the ADTC. For preference, Board ADTC should have at least one member drawn from the PPF or demonstrate the connection between the PPF and the work of the ADTC.

SMC is a consortium of Board ADTCs making advice statements on the clinical and cost-effectiveness of newly licensed medicines. It has been put to me that it is not clear why SMC advice needs to be considered again with the same information by every Board ADTC.

In practice, the Western Isles, Orkney and Shetland share a formulary with Highland and Grampian respectively, Lothian and Borders work closely together, and the regional cancer, diabetes and cardiac networks deliver co-ordinated and agreed treatment clinical pathways, each covering several Health Boards. Thus many decisions are taken collectively now while retaining local ownership and governance. The HIS audit demonstrates also considerable convergence of Board ADTC decision making.

It is claimed by the ABPI that local examination of SMC “accepted” medicines results in inconsistent decisions resulting in unequal access to new medicines, and unnecessary delays citing in their written evidence to the Health and Sport Committee “further hurdles before the medicine reaches patients”. The specific example cited was national consensus meetings. However further discussion about this example at the Health and Sport Committee Meeting on 18 September 2012 highlighted the complexity for the introduction of the medicine in question on the basis that; there was uncertainty about how this new medicine should be introduced in a safe and effective manner by prescribers who had never used it before. As a result, HIS convened a meeting of relevant specialists from all Boards to agree an implementation plan to ensure that patients accumulated the benefits while Boards managed the prescribing risks. There has only been one such consensus meeting and such a measure represents, in my view, and that of many consultees, good practice to ensure more uniform and equitable uptake of a new medicine for those who might benefit most, exactly countering the criticism above.

#### **RECOMMENDATION 4**

NHS Scotland should consider a national meeting of all relevant specialists, organised by Healthcare Improvement Scotland (HIS) , to agree a national implementation plan for some new medicines accepted by SMC that meet agreed criteria. These criteria may include the introduction of novel, first in class medicines where there is considerable uncertainty of its place in therapy. The plan will apply to all patients covered by the SMC “accepted” and to all Boards to support equity of access. Further, HIS should continue to audit access to new medicines and compliance with CEL 17 (2012) and SGHD/CMO (2012) 1.

The following table sets out some of the advantages and disadvantages of the current system alongside two other models; 3-5 networks or regional ADTC based around existing networks for cancer or heart disease, and a single national ADTC.

**Table 1.**

ADTC model	Advantages	Disadvantages	Costs
Current 14 ADTC	<ul style="list-style-type: none"> <li>• GPs have agreed to follow local Board formularies that they are involved in</li> <li>• Local accountability for resources (10% of budget)</li> <li>• Reflect patients' needs in that community</li> <li>• Prescribers become familiar with medicines and very aware of side effects</li> <li>• Published evidence shows that local ownership correlates with effectiveness</li> <li>• Patients' needs and GP preferences aligned well</li> <li>• Generics adopted rapidly by agreement</li> </ul>	<ul style="list-style-type: none"> <li>• Smaller Boards could pay more for some medicines</li> <li>• Different decisions across Scotland</li> <li>• Access varies across Boards</li> <li>• Variation in the introduction of novel medicines</li> </ul>	<ul style="list-style-type: none"> <li>• GPs do 90% of prescribing</li> <li>• Larger Boards cut deals with wholesalers</li> <li>• CHPs use pricing and GP incentives to make significant drug savings in year</li> <li>• Costs managed well locally</li> <li>• Opportunity costs of consultant and GP time</li> <li>• Costs reduced through generics</li> </ul>
Network ADTC (3-5) reflecting existing networks and cross-Board referrals	<ul style="list-style-type: none"> <li>• Agreement across Boards for new, novel medicines, and works already in existing clinical networks</li> <li>• May improve access for some patients</li> <li>• New purchasing arrangements may reduce costs</li> </ul>	<ul style="list-style-type: none"> <li>• Delays in getting specialty groups together</li> <li>• High risk of GP disengagement</li> <li>• Reduce Board and CHP flexibility</li> </ul>	<ul style="list-style-type: none"> <li>• Increased transactional costs</li> <li>• High risk of GP prescribing budgets being exceeded</li> </ul>
Single ADTC for Scotland	<ul style="list-style-type: none"> <li>• Hard to distinguish from SMC</li> <li>• Access will be delayed for all new hospital medicines but may be more uniform</li> <li>• New purchasing arrangements to control overall costs</li> <li>• Should improve perception of better access for all and across Scotland</li> </ul>	<ul style="list-style-type: none"> <li>• Very high risk of GP disengagement with more off formulary prescribing</li> <li>• GPs will develop practice based formularies to control costs</li> <li>• High risk of consultant disengagement</li> <li>• Delays in access</li> </ul>	<ul style="list-style-type: none"> <li>• Increased costs of medicines</li> <li>• Increased opportunity and transactional costs to Boards</li> <li>• No flexibility at Board or CHP for managing large budget</li> <li>• Higher</li> </ul>

		<ul style="list-style-type: none"> <li>• No local accountability for medicines use</li> <li>• Goes against published evidence on the effectiveness of local formularies</li> </ul>	<p>opportunity costs for GPs and consultants</p> <ul style="list-style-type: none"> <li>• NHS Boards unable to manage properly a significant 10% of budget</li> </ul>
--	--	--	---

ADTC do more than maintain a local formulary of medicines based on SMC recommendations. They act as a catalyst for education in the correct use of medicines and therapeutic developments, and agree many other aspects of medicines management including intravenous medicines, special medicines for skin disease and abdominal illness, feeding supplements, antimicrobial policies based on local laboratory data as well as national policy, medicines surveillance and post marketing identification of side effects. They undertake clinical audit and monitor the compliance with local formulary. These functions are not likely to be replaced by a national body. In my view, the gains from a national ADTC are small in relation to the costs needed and the associated opportunity costs generated. Much more can be done to encourage ADTC to address the issues of variation, delay and lack of transparency as I have recommended. However the ADTC need to work smarter and harder to demonstrate that the local advantages of retaining ADTC are matched by improvements in access to new medicines and public reporting of their work.

### **RECOMMENDATION 5**

NHS Scotland should retain the existing ADTC to maintain alignment of patient and GP interests, safe prescribing and enable Boards to manage their costs. Regional clinical networks could have a role in agreeing equitable access to new medicines in relation to their populations.



## REVIEW OF INDIVIDUAL PATIENT TREATMENT REQUESTS (IPTR)

### INTRODUCTION

NHS Boards are expected to follow SMC advice which is applicable for the majority of patients covered by the medicine's licensed indication. Individual Patient Treatment Requests (IPTR) were introduced in 2010 by CEL 17 (2010). The intention was to ensure that patients, who were different from those in the clinical trials or studies submitted to the SMC as evidence of clinical or cost effectiveness, could access medicines 'not recommended for use' in Scotland by SMC subject to meeting certain clinical criteria. This was a new arrangement to the older notion of 'exceptionality' and recognises that patients are not all the same and that there may be many subtypes of patients/disease that are not included in early clinical trials and submitted study data. The onus was on treating doctors to make the case to a local Panel that treatment for a particular patient was justified because they were different in a clinically significant way from the generality of patients studied in the data submitted to SMC. The patient was expected also to have a significant health gain.

Advice to NHS Boards on the implementation of CEL 17 (2010) were reinforced by SGHD/CMO (2011) 3 and CMO (2012) 1.

### HOW WELL ARE IPTR WORKING?

Reading the evidence provided to the Health and Sports Committee<sup>3</sup> and the Committees Official Reports<sup>4</sup> suggests a mixed picture. There appears to be evidence that IPTRs have been of benefit to many patients across NHS Scotland but evidence also of dissatisfaction. Comments from the public, the pharmaceutical industry, patients, and doctors suggest wide variations in approach to the NHS Board IPTRs across Scotland.

IPTRs were described in evidence to Health and Sport Committee in December 2012, as "not the way to access medicines" not recommended by the SMC. Yet it is clear that some patients do access successfully, though an IPTR, medicines not recommended by the SMC. Much of the discussion focussed around the cost effectiveness threshold, which is a balance between clinical effectiveness and cost. The limited data available shows that there is no systematic bias against cancer drugs in either SMC or IPTR decisions.

---

<sup>3</sup><http://www.scottish.parliament.uk/parliamentarybusiness/CurrentCommittees/52064.aspx>

<sup>4</sup><http://www.scottish.parliament.uk/parliamentarybusiness/CurrentCommittees/29831.aspx>

## **Public**

Evidence from the public representatives I met, newspaper accounts and letters shown to me demonstrate a widespread belief outside the NHS that IPTR are poorly understood and for some represent a convenient back door for any patient to access any medicines not approved for use by the SMC. The clinical nature of the justification for agreeing to the request, and the possibility of refusal, has not been fully understood, and has not been explained to patients when seeking advice. Some public representatives have complained that information about the local implementation of new medicines is not accessible and is not written in plain, understandable language. A good example of useful and clear information is on the Health Rights Information Scotland website <http://www.hris.org.uk/patient-information/information-about-health-services/access-to-new-medicines/> and is available also on the Scottish Government website. However this publication dates from 2010, doesn't mention Area Drugs and Therapeutic Committees (ADTC) or IPTR by name, nor does it give a clear picture of how these Board processes work and how a patient can be involved. Many respondents said that the information was very good but not comprehensive enough.

## **Pharmaceutical industry**

Evidence to the Health and Sports Committee was provided by the Association of British Pharmaceutical Industry in Scotland (ABPI). The industry raised concerns about a lack of consistency and lack of information about IPTR outcomes which they believe indicates a postcode lottery to new medicines of value to selected patients but 'not recommended' by the SMC. Concerns have been expressed also about the perceived lack of access to new medicines, particularly new cancer medicines, leading to a reduction in the research access and spending in Scotland by the pharmaceutical industry. These concerns were echoed by doctors giving evidence to the Health and Sport Committee, but no data was submitted.

The Chief Scientist Office indicate that in recent years there has been a decline in traditional, large phase III commercial trials; this was in line with expectations as the UK is not able to compete on price with emerging markets in Eastern Europe, the Far East and South America. However, Scotland has emerged as a country where smaller phase II studies are placed, frequently involving less than 5 patients, and for studies that are proving difficult to recruit in other countries. As a consequence, the number of commercial trials undertaken at sites in NHS Boards in Scotland held up at over 600 since 2010. Data on contract value from NHS Boards in Scotland suggests there has been no fall off in income for commercial studies.

## **Patients**

Some patients' representatives have given good examples of how the IPTR process has worked for patients. However all have found the process difficult to understand and some feel unsupported throughout a lengthy and uncertain period. I was quoted examples where doctors give a clear explanation to patients and explain the reasons that they are, or are not, different in the sense required for a successful IPTR. Some patients reported however that their doctors either did not understand, or did not support an application. Reasons given to patients by their doctors included lack of experience in the using IPTR, lack of trust in the process locally, or a fatalistic assumption about the outcome. Yet the point of the new medicines arrangements

was to strengthen public understanding and the doctor-patient relationship. Patients should expect a clear explanation and understanding of the benefits and harms of new medicines recommended for use and hence why they might be prescribed or not for them, and the reasons that some new medicines were not recommended and were therefore not available in NHS Scotland. The IPTR was to build upon this collaborative relationship by exploring with patients whether there were good reasons to believe that they might respond to SMC “not recommended” treatments. The reality for many patients appears to fall short of this ideal.

Patients have reported widely differing standards of patient or public involvement in the IPTR process locally, ranging from it being ‘usual’ for patients to attend to no patient attending and from it being ‘usual’ for lay representation on the panel to no lay representation. Some patients feel they have had little opportunity to put their case in writing or in person. The existing guidance published under cover of SGHD/CMO(2011)3 is clear that where patients are able to and wish to participate, they should be given the opportunity to do so. However patients need to understand that the panel considers only clinical factors in making a decision, and attending an IPTR will not impact on the decision-making. Whilst there are confidentiality concerns about having a lay person on the IPTR panel, 2 patients reported to me that their confidence in the impartiality of the process was enhanced by having a lay person in the panel.

## **Doctors**

Many doctors are familiar with the IPTR application and paperwork; they may regard it as a chore but accept that this is how access to SMC ‘not recommended’ medicines is achieved for certain patients based on clinical factors. Doctors indicated support for the IPTR concept but believed that the specialist knowledge required for a successful IPTR was not always available and that there was wide variation in the application of relevant clinical criteria. However some doctors appear to dislike the process because they believe the SMC made the wrong decision and do not believe in or understand a cost threshold based on quality adjusted life years; others do not understand fully the application process or their role clearly; others feel not supported to make a good application. Worryingly, a clinical director told me that some doctors make an application deliberately knowing that it would fail; this seems to me to be dishonest and not a substitution for an honest conversation with the patient.

It was clear to me that those doctors making more than one or two applications each year understood the application process, were supported usually by a pharmacist, had access to the relevant specialist knowledge and were successful more often than not. Doctors who apply occasionally, who lack the relevant specialist knowledge, and who are not supported well by a pharmacist believe their ability to support IPTR for their patients is impaired.

## **Charities**

Evidence from the Health and Social Care Alliance suggested that charities based in Scotland have a good understanding of the IPTR arrangements and can support patients. Charities with their headquarters outside Scotland vary in their understanding and support to the IPTR process. Some, like Diabetes UK, with Scottish branches understand the arrangements but do not have the local resources

to offer patients support in an application; those with no presence in Scotland have little understanding of the new medicines arrangements in Scotland and tend to assume that decisions made by the National Institute for Clinical Excellence (NICE) for England and Wales apply also in Scotland. Patients therefore have a mixed experience of understanding and support from medical charities.

## **Boards**

Arrangements for IPTR vary considerably across NHS Boards. It is clear that the guidance in CEL 17 (2010); SGHD/CMO(2011)3 and SGHD/CMO(2012)1 has not been fully implemented by NHS Boards to achieve consistency of approach. The IPTR paper work is variable and completed in different ways and with different levels of detail. There is inconsistent public involvement or accountability and the involvement of patients is variable too. Training and support for doctors varies considerably, and there is considerable variation in the availability of specialist expertise. One example of good practice is found in the Cancer Centres in Glasgow and Edinburgh; here excellent support is available from senior clinicians, a pharmacist, public health and senior manager. The West and Southeast cancer networks provide excellent support to other Boards in their networks so that patients get the best advice and support wherever they live.

Board IPTR decisions are perceived, like ADTC, to be opaque and to lack accountability to local people and patients. While there are data protection issues to consider, an annual report summary of IPTR decisions across Scotland would go a long way to improving this perception and public involvement would help also with bridging a communication gulf.

### **RECOMMENDATION 6**

All Boards should adopt the same IPTR paperwork and process, based on the examples from Greater Glasgow and Clyde, Lothian or Grampian. The application should contrast the clinical criteria appraised by the SMC where “not recommended” advice has been published with the patient’s disease and personal clinical characteristics so that the reasons for the IPTR are more easily assessed, and can be audited.

### **RECOMMENDATION 7**

The IPTR arrangements in Boards should be audited by HIS to assess compliance with guidance and its consistency of application, and to publish the results.

## **RECOMMENDATION 8**

Clinicians should be provided with basic training and guidance in the IPTR process locally. Clinicians who are uncertain or inexperienced should be able to access specialist advice and support (see recommendation 10).

## **RECOMMENDATION 9**

Boards should consider whether IPTR panels should include a member of the public drawn from the Board's patient and public forum. Member(s) will require training and support.

## **RECOMMENDATION 10**

All doctors considering an IPTR must be able to access consistent, knowledgeable support for their patients. National Services Division (NSD) should establish and maintain a register of approved specialists to support IPTR. One specialist may be sufficient for orphan and ultra-orphan diseases, but more than one specialist may need to be available for more common diseases, or variants, and on a regional basis. The model of the cancer networks is an example.

## **RECOMMENDATION 11**

The Scottish Government and Boards should produce clear and concise documentation, available on national and local websites, that explains the roles of ADTC and IPTR, how the public and patients can be involved, and provide links to the reports recommended above and for ADTC.

## HOW CAN WE SUPPORT PATIENTS BETTER WITH ULTRA-ORPHAN DISEASES?

Rare Diseases UK and others raised concerns that some patients suffering from very rare diseases (known as “ultra-orphan”) may never access effective treatment. Clinicians and their patients regard this as unfair. Ultra-orphan diseases are widely recognised as those occurring in less than 1,000 of the UK population (equating to <100 patients in Scotland).

The pharmaceutical industry has responded positively to European incentives offered to pharmaceutical companies to support the development and licensing of new drugs for the treatment of orphan and ultra-orphan diseases. This is in a context where such medicines will never be widely used and the cost will remain very high always and can be distinguished from other orphan medicines which will treat larger patient numbers where approval is not granted on first application because the initial cost is too high and where the cost could fall because there is a larger market for the medicine or a PAS can be agreed.

The SMC has published advice to confirm its acceptance of some medicines for orphan diseases but not generally those for ultra-orphan diseases. The Patient Access Scheme (PAS)<sup>5</sup> process has contributed to the SMC’s ability to accept some orphan medicines as the discount offered under the terms of the PAS improved their cost-effectiveness, meaning that such medicines are now available within the NHS in Scotland. NHS Board Chief Executives established pooled resources to share the costs and risks of funding high cost, low volume medicines for certain orphan diseases, and a limited number of other treatments e.g. recombinant blood products. The Board of residence contributes their weighted share and any excess costs are covered by the weighted contributions of other Boards to the shared risk pool. This collaborative arrangement has made the decision to provide treatment more equitable and improved access for patients from smaller NHS Boards. NHS Chief Executives exercise their accountability by regular scrutiny of the arrangement, managed on their behalf by the National Services Division (NSD) of National Services Scotland (NSS).

However, some ultra-orphan medicines are not available for NHS Scotland because no application has been made by the manufacturer to the SMC. Among the cited reasons is that there is insufficient data to support a clinical and cost effective application because of the small number of patients treated.

Other ultra-orphan medicines are not approved by SMC because of the uncertainties within the effectiveness evidence submitted or the very high cost, particularly where no PAS has been submitted. Whilst clinicians in Scotland have made IPTR applications for treatment; patient interest groups report variation in IPTR decisions being reached even where the clinical circumstances of the patients concerned appear to be very similar. These concerns add to the perception of inconsistent application of the IPTR process. This is a source of frustration and anger among

---

<sup>5</sup> Patient Access Schemes represent a discount arrangement to facilitate the entry of new medicines and managed by National Services Scotland (NSS) through assessment of the scheme via the Patient Access Scheme Assessment Group (PASAG).

patients and patient interest groups. I therefore made an interim recommendation to the Cabinet Secretary for Health and Wellbeing that additional funding should be made available to address these issues.

## RARE CONDITIONS MEDICINES FUND – A RECOMMENDED APPROACH

The Cabinet Secretary for Health and Wellbeing announced on 14 January 2013, the establishment of a Rare Conditions Medicines Fund (RCMF) to cover the cost of medicines for individual patients with rare conditions following a successful new Individual Patient Treatment Request. The fund is available from 1 March 2013 for a period of 13 months<sup>6</sup>.

Patients with ultra-orphan conditions may be looked after by a variety of clinicians, often supported by a single specialist in Scotland or elsewhere. Applications for SMC “not recommended” ultra-orphan medicines would need to demonstrate failure to respond to current treatment, or no other alternative, as well as a significant expected clinical benefit greater than that experienced by the trial population and within a reasonable time frame.

The starting position for accessing the RCMF is where the SMC has published advice to confirm that a new medicine to treat an ultra-orphan condition has been “not recommended” for use following a full submission. Access to the Rare Conditions Medicine Fund (RCMF) recommended above would be considered following a successful IPTR application for a single patient. Where such circumstances apply to a cohort of patients, decisions regarding the need to consider Group Patient Treatment Requests (GPTR) for particular orphan medicines will be based on specialist clinical advice and agreed at a national level.

For both IPTR and GPTR, it would be essential to show that the medicine had a reasonable expectation of being clinically effective and was for an ultra-orphan disease. It is important that the process to consider the clinical circumstances for IPTR or GPTR applications is supported by the transparent and consistent assessment of the clinical context of the patient(s) by a nominated and recognised specialist in Scotland, or elsewhere. It is also important to be clear that whether or not the medicine has the potential to qualify for RCMF funding should not be a consideration of the decision-making panel.

Based on my interim advice to the Cabinet Secretary, I recommend some principles for access to RCMF funding:

- a full submission had been made to the Scottish Medicines Consortium for the orphan or highly specialist medicine;
- the request for the medicine has been subject to successful new IPTR or GPTR panel decision (i.e. the Fund will not be applied retrospectively);
- the request for the medicine’s use is within its licensed indication; and
- a clinical judgement has been made that the patient (or patients) have the capacity to derive clinical benefit from the orphan medicine (with the caveat that decisions regarding the need to consider Group Patient Treatment Requests

---

<sup>6</sup><http://www.scotland.gov.uk/News/Releases/2013/01/medicines-fund14012013>



(GPTR) for particular orphan medicines will be based on specialist clinical advice and agreed at a national level).

The applications and outcomes for IPTRs funded through this mechanism should be monitored for completeness and consistency of the process by NSD. The Rare Conditions Medicines Fund should be managed by NSD on behalf of NHS Boards in a similar manner to the risk pooling arrangement described. NSD would monitor expenditure and report regularly to Chief Executives.

## **RECOMMENDATION 12**

The RCMF should focus on access to medicines for ultra-orphan diseases. Access should be supported where the SMC has published 'not recommended' advice after a full submission of the medicine, **and** after a successful IPTR or GPTR has been agreed.

Prof C P Swainson, FRCPE, FRCSE (Hon), FFPHM

April 2013

### NEW MEDICINES REVIEW

The New Medicines Review announced by the Cabinet Secretary for Health and Wellbeing announced on the 14 November 2012 responds to concerns raised in the Scottish Parliament and the media regarding concerns of patients, clinicians and other stakeholders regarding access to new medicines – particularly cancer medicines and medicines to treat rare diseases.

The New Medicines Review will examine every aspect of the processes for introducing new medicines within the NHS in Scotland to assess their effectiveness and to identify what further improvements can be made.

The review comprises three main strands of work:

- (iv) an independent examination of the Scottish Medicines Consortium (SMC) appraisal processes; **(Annex A)**;
- (v) an examination of the role and remit of NHS Board Area Drug and Therapeutics Committees (ADTCs) and a separate examination of the current Individual Patient Treatment Request (IPTR) arrangements **(Annex B)**; and
- (vi) an audit of NHS Board formulary decisions regarding medicines which have been accepted or accepted for restricted use by the SMC (Annex C).

A key aim of the review will be to achieve consistency in the application of national policy throughout NHSScotland. A fuller description and the scope of each strand of work are set out in the attached annexes.

The New Medicines Review will report early in 2013.

Pharmacy and Medicines Division

## **Review of the Scottish Medicines Consortium (SMC) Processes**

### Description and Scope

Professor Philip Routledge, Professor of Clinical Pharmacology at Cardiff University and Mrs Karen Samuels, Programme Director for the All Wales Therapeutics and Toxicology Centre will examine the current Scottish Medicines Consortium (SMC) appraisal processes and methodology from horizon scanning of new pharmaceutical products in the pipeline through to provision of SMC advice to NHSScotland to see what further improvements can be made.

This will include a broader view of the cost and benefits of new, innovative medicines – specifically to assess whether the Quality Adjusted Life Year (QALY) methodology represents an effective tool to calculate cost-effectiveness of these in terms of off-setting the cost of the medicine against potential savings when they displace existing treatment(s) and prevent possible hospitalisation.

### Timeframe

The Review will commence on 5 December 2012 and Professor Routledge and Mrs Samuels will report on their findings to the Chief Pharmaceutical Officer of the Scottish Government early in the New Year.

## **Refresh of NHS Board Area Drug and Therapeutics Committee Short Life Working Group (SLWG)**

### Description and Scope

Professor Charles Swainson will chair a Short Life Working Group (SLWG) to refresh and agree the role and remit of NHS Board ADTCs, building on and strengthening the existing work and structures in NHS Boards.

This will include an examination of whether there is a need for 14 sets of criteria for prescribing medicines which have been accepted for use by the Scottish Medicines Consortium (SMC) and what further improvements can be made to achieve consistency of application of national policy across NHSScotland.

### Output

The findings of the SLWG will be reported to the Chief Pharmaceutical Officer of the Scottish Government.

### Timeframe

The SLWG will report on their findings early in the New Year.

## **Review of NHS Board Management of Individual Patient Treatment Requests (IPTRs)**

### Description and Scope

Professor Charles Swainson will examine the extant Individual Patient Treatment Request (IPTR) arrangements and provide comment on:

- their effectiveness – specifically whether this is a reasonable approach in relation to orphan medicines and cancer medicines;
- the benefits of establishing a single national protocol for consideration of IPTRs across NHSScotland; and
- whether there is scope for further improvements to the existing arrangements including consistency in the application of national policy.

### Output

Professor Swainson will report his findings to the Chief Pharmaceutical Officer of the Scottish Government.

### Timeframe

Professor Swainson will report on his findings early in the New Year.

## **Implementation of SMC “Accepted” Advice within NHS Boards in Scotland**

### Description and Scope

Healthcare Improvement Scotland will undertake an audit of medicines which have been accepted or accepted for restricted use by the SMC for a 5 month period and will examine for each:

- the decision taken on each medicine by each NHS Board;
- the date of the decision; and
- the date the decision was published on the NHS Board website

The timescales for making the decision and publishing it will be measured against the timelines contained in the Scottish Government guidance document published under cover of SGHD/CMO(2012)1 on 13 February 2012 which took effect from 1 April 2012.

Accessibility of standard advice about formulary decisions and rationale for these for patients and the public will also be assessed for each NHS Boards to ensure they are complying with the guidance.

### Output

The output from this piece of work will help support the New Medicines Review and will be provided to the Chief Pharmaceutical Officer of the Scottish Government.

### Timeframe

Healthcare Improvement Scotland will report on their findings early in the New Year.

### Refresh of Area Drugs and Therapeutics Committees (ADTC)

#### BACKGROUND

ADTCs are key to ensuring that adequate systems and processes relating to medicines governance are in place in local NHS Boards. They are clinically led and clinically driven committees ensuring medicines issues are addressed across the health system.

The last guidance issued relating to the function and roles of ADTC was issued in the early 1990s. The Quality Strategy, Health and Social Care Integration agenda require us to refresh that guidance so that it is fit for purpose.

#### PURPOSE and FUNCTIONS

To provide **professional advice, clinical advice** and **leadership** to the NHS Board, that supports safe, clinically effective, cost effective and patient centred **medicines governance**, in all care settings.

Examples of this include

- developing, maintaining and/or promoting policies and systems for safe and secure use of medicines (e.g. Unlicensed and off-label medicines, recording and management of medicines)
- providing clear direction and delivery in relation to the NHS Board formulary following Scottish Medicines Consortium advice, promoting safe and cost effective medicines use
- monitoring trends, analysis and dissemination of learning from medication incidents
- participating in the Yellow Card Scheme for reporting adverse drug reactions.

To advise and support the **strategic direction** of all aspects of **medicines governance** and usage in all care settings ensuring inclusion within wider strategic planning carried out by the NHS Board.

Examples of this include

- supporting Antimicrobial Stewardship through the work of the Scottish Antimicrobial Prescribing Group (SAPG) via the Antimicrobial Management Teams
- supporting the NHS Board in meeting its statutory responsibilities in relation to medicines and prescribing
- supporting the NHS Board in the delivery of a comprehensive approach to national policy regarding medicines, linking with Regional and National groups where appropriate.
- Supporting the eHealth agenda and Scottish Patient Safety Programme and Significant Adverse Events

To ensure **multi-stakeholder engagement** and joint working on all medicine related issues within **all care settings**, including social care settings.

Examples of this include

- Developing, in conjunction with Communication teams, internal and external communication strategies for the public, patients and health care professionals regarding medicines use in the Board area
- Involving members of the public in the work of the ADTC
- Supporting the engagement of clinicians in initiatives to develop, implement and monitor systems to ensure seamless care for patients at the transition points of admission and discharge from hospital e.g. Quality Hub, Quality Improvement Teams
- Developing policies to support safe and effective use of medicines with social care partners.
- Through multi-stakeholder engagement ensuring that medicines are used safely in all community settings
- Working with the pharmaceutical industry in line with “A Common Understanding”.
- Contributing to learning and safe practice through liaison with under graduate and post graduate tutors in relation to local educational initiatives to improve medicine use
- Promoting safe practice through liaison with R and D and audit committees to improve medicine use
- Publishing all information in an accessible format
- Providing end of year report to the Board.

To inform the **financial planning and governance** of the NHS Board to ensure the effective use of resources, in relation to **medicines**.

Examples of this include

- Contributing to effective horizon scanning to ensure the NHS Board have information on new medicines
- Advising on resource implications on the introduction of new medicines.
- Co-ordinating the qualitative review of medicines use, and providing clinical information on changing trends in medicines use.

## **REPORTING ARRANGEMENTS**

The ADTC is the key professional advisory group for medicine governance and will report into the NHS Board via the Board’s clinical governance structure.

The Director of Pharmacy is responsible for medicine governance; with a line management responsibility from Director of Pharmacy to Medical Director and Chief Executive Officer to NHS Board.

The NHS Board needs to define what functions, if any, are directly delegated to the ADTC.

NHS Board ADTCs will network to share good practice and develop consistent policies where appropriate.

## **MEMBERSHIP**

The membership should predominantly include clinical practitioners with an interest in medicines use within the Board. The Board should appoint the Chair\*.

Membership should be open to local flexibility and be commensurate with the size and needs of the NHS Board but as a core would include:

Chair\* (Consultant, General Practitioner, lead Pharmacist or non-executive director)  
Professional Secretary  
Director of Pharmacy  
Administrative support (from the Board Secretariat)  
Hospital Consultants (from a range of clinical specialities)  
General Practitioners  
Pharmacists (representing both Hospital and Community)  
Nurses

NHS Boards may wish to engage with other stakeholders as required on specific work streams or to extend membership to include; Dentists, Social care staff, Executive Medical Director, Executive Nurse Director, Managers, Allied Health Professionals, Finance and Academia/ Research, Clinical Governance Leads.

There should be cross representation with other key related workstreams such as Scottish Patient Safety Programme (SPSP).

The ADTC must be able to demonstrate effective patient and public engagement.

## **SUB GROUPS**

The Antimicrobial Management Team (AMT) is a nationally recognised sub group of the ADTC.

ADTC may wish to develop further Sub Groups to take forward specific areas within the overall remit. Where these are convened they should have delegated authority to act on behalf of the ADTC, reporting and referring upwards as required.



---

## **Monitoring implementation of SGHD/CMO(2012)1**

April 2013

## Contents

Executive summary .....	66
1 Introduction .....	67
2 Methodology .....	67
3 Results.....	68
4 Discussion .....	70
Appendix 1: Medicines approved by SMC April-September 2012.....	73
Appendix 2: Web search criteria.....	75
Appendix 3: Breakdown of NHS boards' ADTC decisions.....	76
Appendix 4: NHS board decisions for each medicine.....	77
Appendix 5: NHS board compliance per board for decision-making and timeliness ....	79
Appendix 6: CMO(2012)1 guidance example list of SMC medicines and formulary decisions.....	80
Appendix 7: Individual NHS board feedback .....	81
References .....	87

## Executive summary

This report details the compliance of NHS boards in Scotland with the SGHD/CMO(2012)1. Twelve\* NHS boards in Scotland responded, within tight timescales, to the request for data and subsequent requests to validate the data. This is to be commended.

All respondents had made a decision on all of the 23 medicines reviewed and the average percentage uptake of SMC accepted medicines on to the 12 boards' formularies was 74%. Of those not included, the most common reason given by the Area Drug and Therapeutic Committees (ADTCs) is that clinicians did not support formulary inclusion. Ten NHS boards used the CMO framework to categorise their decisions. In addition, 87% of all the decisions made by all NHS boards on all medicines were made within the required 90 days, with four boards making 100% of its decisions in 90 days.

In investigating the subsequent reporting of these local decisions, we identified that 70% of the NHS boards published their decisions within the required 14 days. Given the timing of the data collection exercise immediately after the introduction of the CMO, this suggests a relatively rapid adoption of the requirement to publish, with most NHS boards choosing to present their information as defined.

Using a series of search terms, we independently sought to determine how accessible the local ADTC decisions are. We identified that, although there appears to be an acceptance of the requirement to publish the decisions, the relevant websites or web pages are not as well signposted as they could be. This may reflect a need for wider tagging and better insight into how members of the public might choose to search.

This is the first time that it has been possible to report on the uptake of SMC medicines across NHS boards, the timeframe of these decisions and their publication. The principles of the CMO guidance have largely been accepted across NHSScotland; local decision making on new medicines is overall both timeous and publicised. Due to their specialist nature, the differences in local service provision for specialist conditions and the fact that an "equivalent"<sup>2</sup> was already available on the formulary, justifiable variation in decisions made between NHS boards for the 23 medicines reviewed was demonstrated. Scope for improvement includes the consistent application of categories, improved accessibility of the information on the websites where decisions are posted, and a faster response rate for some ADTCs to make and publish the decisions.

A series of recommendations have been given to the Scottish Government and individual feedback has been provided to each NHS board.

\* 12 boards responded, however two NHS boards follow the advice of mainland boards and therefore did not participate in the data collection exercise.

## 1 Introduction

On 13 February 2012, Scottish Government published SGHD/CMO(2012)1 *Guidance to Further Strengthen the Safe and Effective use of New Medicines Across the NHS in Scotland*<sup>1</sup>, which detailed further measures to strengthen previous guidance issued under CEL 17 (2010)<sup>2</sup> and SGHD/CMO(2011)3<sup>3</sup>.

One of the key purposes of CMO(2012)1 was to “standardise a timeframe for NHS boards to consider Scottish Medicines Consortium (SMC) accepted medicines and to publish advice accordingly”<sup>1</sup>.

In August 2012, the Chief Pharmaceutical Officer at Scottish Government asked Healthcare Improvement Scotland to undertake a data collection exercise. This first data collection exercise was undertaken to understand the extent to which SMC advice (for medicines approved during 2011) had been implemented in the 14 territorial NHS boards. This data collection exercise was on SMC advice issued prior to the requirements of CMO(2012)1, therefore, aggregation of data across NHSScotland to describe the nature of ADTC decisions was not possible due to variation in the terminology used.

As part of the New Medicines Review commissioned by the Chief Pharmaceutical Officer, Healthcare Improvement Scotland was asked to repeat the data collection exercise, specifically auditing NHS board compliance with CMO (2012)1.

## 2 Methodology

Letters from our Chief Pharmacist were sent to the NHS boards, informing them of the repeat data collection exercise and including each NHS board’s specific feedback from the first data collection exercise.

A list of medicines ‘accepted’ or ‘accepted for restricted use’ by SMC from April to September 2012 was compiled, totalling 23 medicines (detailed in Appendix 1). This was the SMC advice issued during the first 5 months following the CMO guidance.

This list of medications was distributed to each of the 14 NHS boards, through their formulary pharmacist and/or director of pharmacy, with a request to report on the following;

1. the decision taken by each NHS board’s area drug and therapeutic committee (ADTC),
2. the date of the decision (the date must be within 90 days of the SMC advice to the NHS boards), and
3. the date this decision was published on the NHS board’s website (the date must be within 14 days of the NHS board decision).

Simultaneously, we tried to source the above information from individual NHS boards’ websites to determine accessibility of that information to patients and the public. A list of search terms (detailed in Appendix 2) was used to interrogate the websites.

Once the above data had been collated, individual feedback reports were then sent to each of the 14 NHS boards, requesting them to validate their submitted data by return.

### 3 Results

The findings below detail the figures from the three key questions.

Twelve boards responded with complete data. The two NHS boards that did not provide data follow the decisions of NHS Grampian's ADTC; therefore their data duplicates NHS Grampian's.

For the purposes of this report, the data from the 12 NHS boards that responded will be used in the analysis.

Also included is information on the accessibility of information of new medicines from each NHS board's website.

#### 3.1 What was the decision taken on each medicine by each NHS board?

All 12 boards made a decision on all 23 medicines.

The average percentage uptake of SMC accepted medicines on to the 12 NHS boards' formularies was 74% (17 out of 23) (decision categories 1 and 2). One of the 12 NHS boards included all 23 medicines on to their formulary.

CMO(2012)1 detailed the following categories for formulary decisions. Ten out of the 12 NHS boards used the categories below to indicate their ADTC decisions.

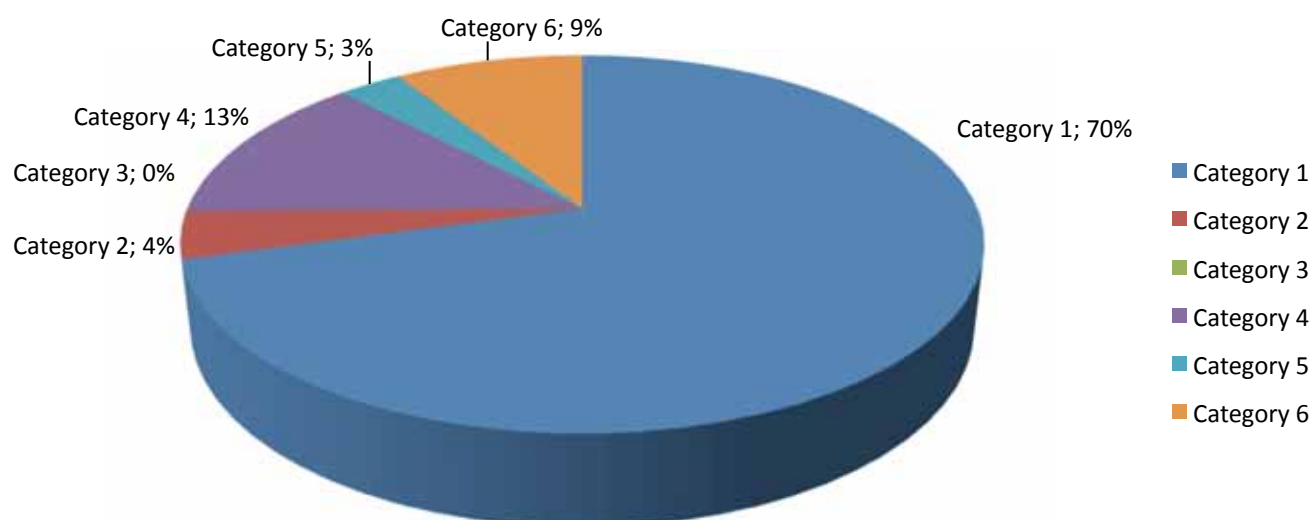
1. **Included** on the NHS board formulary (**to include specialist, approved, additional lists**) for the indication in question.
2. **Included** pending protocol.
3. **Not included\*** from the NHS board formulary because the NHS board decision is that the medicine does not represent sufficient added benefit to other comparator medicines to treat the condition in question which are already available in the formulary.
4. **Not included\*** from the NHS board formulary because clinicians do not support the formulary inclusion.
5. **Not included\*** from the NHS board formulary because clinicians have not responded to an invitation to apply for formulary inclusion for this medicine.
6. **Not included\*** pending protocol.

\* Where a medicine has not been included in the formulary, there will be a link to the formulary in order that the comparator medicines can be viewed.

Appendix 3 details the breakdown of each NHS board's decisions on the 23 medicines.

Figure 1 below illustrates the breakdown of ADTC decision categories used by the NHS boards for all 23 medicines.

**Figure 1: Breakdown of NHS boards' use of ADTC decision categories**



### **3.2 Was the decision taken by the ADTC within 90 days?**

The NHS boards made an average of 87% (20 out of 23) of their decisions on all 23 newly licensed medicines within 90 days (see Appendix 5). Four boards made 100% (23 out of 23) of their decisions on all 23 newly licensed medicines within 90 days. One board made 96% (22 out of 23) and two boards made 91% (21 out of 23) of their decisions on the 23 medicines within 90 days. The remaining five boards made between 57% (13 out of 23) and 78% (18 out of 23) of their decisions within 90 days.

Four medicines in particular did not have their decisions made on them within 90 days, across five boards.

### **3.3 Was the decision published on the NHS boards' website?**

On average, 70% (16 out of 23) of ADTC decisions were published by the boards, on their websites, within 14 days of their ADTC decisions being made.

Four NHS boards published decisions of all 23 medicines on their board website within 14 days of the ADTC decisions being made. One board published 91% (21 out of 23) of their ADTC decisions on to their board website within 14 days. Six boards published between 26% (6 out of 23) and 83% (19 out of 23) of their ADTC decisions on their website within 14 days of their ADTC decisions being made. One board did not provide dates of publication for their ADTC decisions (see Appendix 5).

### **3.4 How accessible was this information to patients and the public?**

We viewed all 14 NHS boards' websites to determine accessibility for patients and the public on the decisions made by their own board's ADTC, using the search terms detailed in Appendix 2.

CMO(2012)1 guidance outlines the type of list that NHS boards are expected to maintain on their website (see Appendix 6), in relation to SMC medicines and formulary decisions. NHS boards are expected to publish updated lists of SMC accepted medicines **included** and **excluded** from their formularies, together with the rationale for such decisions.

It was found that seven boards had easy access for the public to see ADTC decisions and patient information, and allowed access in a logical and intuitive way to the boards' formularies. The information was not always presented in accordance with CMO(2012)1.

For three boards, their access for the public was somewhat limited. For example, one board had an external formulary website that gave excellent information on local formulary decisions, but nothing linking the main board website to the external site. This means patients or members of the public would only be able to find the information if searched for directly in a search engine, such as Google.

The remaining four boards had no information relating to their formularies and had no information available regarding ADTC decisions. When conducting a search, using the search terms detailed in Appendix 2, none of these boards provided a link to their formularies, and in the case of two boards, did not have a search function on their websites.

## **4 Discussion**

CMO(2012)1 has introduced a common framework for NHS boards to adopt that should allow NHS boards to apply common principles and processes in the introduction of newly licensed medicines to facilitate consistency of approach to local decision-making.

### **4.1 The decision taken for each medicine by each NHS board**

The majority (74%) of SMC accepted medicines were included in NHS board formularies. Of those not included, the most common reason given by ADTCs is category 4: "clinicians did not support formulary inclusion". For this decision category, 50% of the boards confirmed that clinicians did not support formulary inclusion because an "equivalent"<sup>2</sup> was available, those medicines were not required in their board area because they do not have the applicable specialist clinics or those medicines are only used by specialist clinicians and are therefore not applicable to the general formulary. The remaining 50% of boards that used category 4 did not provide a rationale for why clinicians did not support inclusion on to their boards' formularies.

Some of the variation between board ADTCs about which medicines are included on their formularies is explained by the differing local needs (for example, specialist paediatric medicines are only required in tertiary centres). When a medicine is not included by more than one board's ADTC, there is variation in the reasons given for why the medicine has not being included. This variation needs to be further explored with the boards as it may reflect different local service provision or inconsistent use of the decision categories.

The previous data collection exercise in 2012 was undertaken before the introduction of the CMO guidance. This prevented us from being able to comment on uptake of SMC accepted medicines across the boards, due to the variability of their data. The categories from CMO(2012)<sup>1</sup> have since provided the boards with a consistent framework to report their ADTC decisions and subsequently helped us to determine the spread of uptake of medicines across the boards. The CMO guidance has also helped boards to describe the implementation of SMC advice in their board area. Appendix 4 details the breakdown of each medicine and which ADTC decision category they were given by the boards.

#### **4.2 Decisions made within 90 day target**

Of the 23 medicines audited, all except three (exenatide, fidaxomicin, colecalciferol) are considered highly specialised medicines. The highly specialised nature of these medicines and the service provision required could account for the delay in most boards not making all of their ADTC decisions within 90 days, as specialised expertise input would be required. For example, of the above three medicines, fidaxomicin, a non specialised medicine, was included on to the formularies of 10 out of the 12 boards and ADTC decisions were made on this drug within 90 days by all of the 12 boards.

The five medicines that did not make the 90 day target were all related to advice from SMC in April and May, immediately after the guidance was issued. The time taken for the boards to create and update local processes to facilitate compliance with the CEL may explain this delay.

Also, there may be differences in the frequency of ADTC meetings, which could cause decisions on specialist medicines to be delayed significantly if specialist input is not available.

#### **4.3 Decision was published on the NHS board website**

The audit found that most boards were relatively timely in adding their ADTC decisions to their websites. With the exception of three boards (and one board that did not provide dates of publication), the boards published at least 70% (16 out of 23) of their decisions within 14 days of their ADTC decisions being made.

This is a marked improvement on the data provided by the boards for the baseline data collection exercise in 2012, which highlighted that prior to the introduction of the CMO guidance, there was no requirement for the boards to present this information on their websites.

#### **4.4 Transparency of decision making**

From the audit on all 14 NHS boards, we can conclude that ADTC decisions are largely being made transparent, with only four boards failing to provide sufficient access to the board formularies or a note of ADTC decisions on their websites. Of the seven boards that had excellent access to view ADTC decisions and the board formularies, three published their ADTC decisions in a format consistent with the CMO(2012)<sup>1</sup> guidance (see Appendix 6).



However, for patients and the public, the search terms required to access this information may not be intuitive and, therefore, the accessibility of the published information may be more challenging.

## Appendix 1: Medicines approved by SMC April-September 2012

Drug Name	Publication date	SMC ID	Manufacturer	Status
<a href="#">Everolimus (Afinitor)</a>	06/04/2012	777/12	Novartis Pharmaceuticals UK Ltd	Accepted
<a href="#">Insulin detemir (Levemir)</a>	06/04/2012	780/12	Novo Nordisk	Restricted
<a href="#">Etanercept (Enbrel)</a>	06/04/2012	781/12	Wyeth Pharmaceuticals	Restricted
<a href="#">Etanercept (Enbrel)</a>	06/04/2012	782/12	Wyeth Pharmaceuticals	Restricted
<a href="#">Collagenase (Xiapex)</a>	16/04/2012	715/11	Pfizer	Restricted
<a href="#">Pregablin oral solution (Lyrica)</a>	04/05/2012	765/12	Pfizer	Restricted
<a href="#">Alteplase (Actilyse)</a>	04/05/2012	714/11	Boehringer Ingelheim Ltd	Accepted
<a href="#">Tobramycin inhalation powder 28mg (TOBI Podhaler)</a>	04/05/2012	783/12	Novartis Pharmaceuticals	Accepted
<a href="#">Dexmedetomidine hydrochloride (Dexdor)</a>	04/05/2012	784/12	Orion Pharma	Accepted
<a href="#">Exenatide twice-daily in combinaton with insulin (Byetta)</a>	04/05/2012	785/12	Lilly UK	Accepted
<a href="#">Dexamethasone (Ozurdex) 0.7 mg injecton</a>	04/05/2012	652/10	Allergan	Restricted
<a href="#">Fidaxomicin (Dificlir)</a>	08/06/2012	791/12	Astellas Pharma Ltd	Restricted
<a href="#">Eplerenone (Inspra)</a>	08/06/2012	793/12	Pfizer UK	Accepted
<a href="#">Tadalafil (Adcirca)</a>	08/06/2012	710/11	Eli Lilly & Co. Ltd	Restricted
<a href="#">Golimumab (simponi)</a>	08/06/2012	674/11	MSD/Schering Plough	Restricted
<a href="#">Pegylated interferon alfa 2b (Viraferon Peg)</a>	08/06/2012	794/12	Merck Sharp & Dohme Ltd	Accepted
<a href="#">Rufinamide 40mg/mL oral suspension (Inovelon®)</a>	08/06/2012	795/12	Eisai Ltd	Restricted
<a href="#">Abiraterone (Zytiga)</a>	06/07/2012	764/12	Janssen-Cilag Ltd	Restricted
<a href="#">Mercaptopurine (Xaluprine)</a>	06/07/2012	798/12	Nova Laboratories Limited	Accepted

<b>Drug Name</b>	<b>Publication date</b>	<b>SMC ID</b>	<b>Manufacturer</b>	<b>Status</b>
<a href="#">Fingolimod (Gilenya)</a>	10/08/2012	763/12	Novartis Pharmaceuticals UK Ltd	Restricted
<a href="#">Tocilizumab (RoActemra)</a>	10/08/2012	774/12	Roche	Restricted
<a href="#">Colecalciferol (Fultium-D3)</a>	10/08/2012	801/12	Internis Pharmaceuticals Ltd	Accepted
<a href="#">Tegafur/gimeracil/oteracil (Teysono)</a>	10/08/2012	802/12	Nordic Pharma Ltd	Restricted

## **Appendix 2: Web search criteria**

To determine accessibility to patients and the public of information on new medications and the process undertaken for these decisions, the below search terms were identified, which included terms it was assumed a member of the public would understand, specific medicine names from the above list, and terms that would require some knowledge of the process for new medicines. The terms used were;

- Medicines
- Fidaxomicin
- Everolimus
- Tocilizumab
- Colecalciferol
- Prescribing
- SMC
- Formulary
- ADTC

These terms were entered into each NHS board's main website's search facility, in the order presented above, and any information regarding decisions taken on new medicines recorded.

### Appendix 3: Breakdown of NHS boards' ADTC decisions

The table below Indicates each board's breakdown of medicines into the six decision categories

Board	ADTC decision categories*					
	1	2	3	4	5	6
NHS A&A	15 (65%)	2 (9%)	0 (0%)	2 (9%)	1 (4%)	3 (13%)
NHS Borders	17 (74%)	0 (0%)	1 (4%)	1 (4%)	0 (0%)	4 (17%)
NHS D&G	8 (35%)	5 (22%)	3 (13%)	0 (0%)	3 (13%)	4 (17%)
NHS Fife	21 (91%)	0 (0%)	0 (0%)	1 (4%)	1 (4%)	0 (0%)
NHS FV	20 (87%)	0 (0%)	0 (0%)	3 (13%)	0 (0%)	0 (0%)
NHS Grampian	14 (61%)	1 (4%)	0 (0%)	2 (9%)	6 (26%)	0 (0%)
NHS GG&C	20 (87%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	3 (13%)
NHS Highland	15 (65%)	0 (0%)	0 (0%)	8 (35%)	0 (0%)	0 (0%)
NHS Lanarkshire	23 (100%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)
NHS Lothian	11 (48%)	2 (9%)	0 (0%)	3 (13%)	3 (13%)	4 (17%)
NHS Orkney	No Data Provided - as per NHS Grampian					
NHS Shetland	No Data Provided - as per NHS Grampian					
NHS Tayside	18 (78%)	0 (0%)	0 (0%)	3 (13%)	0 (0%)	2 (9%)
NHS WI	15 (65%)	0 (0%)	0 (0%)	8 (35%)	0 (0%)	0 (0%)
<b>Average</b>	<b>16 medicines</b>	<b>1 medicines</b>	<b>0 medicine</b>	<b>3 medicines</b>	<b>1 medicine</b>	<b>2 medicines</b>
<b>Average %</b>	<b>70%</b>	<b>4%</b>	<b>0%</b>	<b>13%</b>	<b>4%</b>	<b>9%</b>

#### \* ADTC decision categories:

1. **Included** on the NHS board formulary (**to include specialist, approved, additional lists**) for the indication in question.
2. **Included** pending protocol.
3. **Not included\*** from the NHS board formulary because the NHS board decision is that the medicine does not represent sufficient added benefit to other comparator medicines to treat the condition in question which are already available in the formulary.
4. **Not included\*** from the NHS board formulary because clinicians do not support the formulary inclusion.
5. **Not included\*** from the NHS board formulary because clinicians have not responded to an invitation to apply for formulary inclusion for this medicine;
6. **Not included\*** pending protocol.

## Appendix 4: NHS board decisions for each medicine

The table below illustrates the count of boards for each decision category, for each medicine.

Medicine	ADTC Decision Categories*						% of boards that	
	1	2	3	4	5	6	Included this drug on formularies	Excluded this drug on formularies
Insulin detemir (Levemir)	12	0	0	0	0	0	100%	0%
Mercaptopurine (Xaluprine)	12	0	0	0	0	0	100%	0%
Tocilizumab (RoActemra)	11	1	0	0	0	0	100%	0%
Etanercept (Enbrel) (782/12)	11	0	1	0	0	0	92%	8%
Pregablin oral solution (Lyrica)	11	0	0	0	1	0	92%	8%
Exenatide (Byetta)	11	0	0	0	1	0	92%	8%
Abiraterone (Zytiga)	11	0	0	0	0	1	92%	8%
Colecalciferol (Fultium-D3)	10	1	0	0	0	1	92%	8%
Everolimus (Afinitor)	10	0	1	0	0	1	83%	17%
Etanercept (Enbrel) (781/12)	10	0	1	0	1	0	83%	17%
Alteplase (Actilyse)	10	0	1	0	1	0	83%	17%
Eplerenone (Inspra)	9	1	0	0	1	1	83%	17%
Fidaxomicin (Dificlir)	8	2	0	0	0	2	75%	25%
Tadalafil (Adcirca)	8	1	0	3	0	0	75%	25%
Rufinamide (Inovelon®)	8	1	0	2	1	0	75%	25%
Fingolimod (Gilenya)	9	0	0	0	1	2	75%	25%
Tobramycin (TOBI Podhaler)	8	0	0	2	1	1	67%	33%
Dexamethasone (Ozurdex)	7	1	0	0	2	2	67%	33%
Pegylated interferon alfa 2b (Viraferon Peg)	6	1	0	2	2	1	58%	42%
Dexmedetomidine hydrochloride (Dexdor)	6	0	0	2	1	3	50%	50%
Collagenase (Xiapex)	5	0	0	3	2	2	42%	58%
Golimumab (simponi)	5	0	0	6	0	1	42%	58%
Tegafur/gimeracil/oteracil (Teysuno)	2	0	0	7	1	2	17%	83%

**\* ADTC decision categories:**

1. **Included** on the NHS board formulary (**to include specialist, approved, additional lists**) for the indication in question;
2. **Included** pending protocol;
3. **Not included\*** from the NHS board formulary because the NHS board decision is that the medicine does not represent sufficient added benefit to other comparator medicines to treat the condition in question which are already available in the formulary;
4. **Not included\*** from the NHS board formulary because clinicians do not support the formulary inclusion;
5. **Not included\*** from the NHS board formulary because clinicians have not responded to an invitation to apply for formulary inclusion for this medicine;
6. **Not included\*** pending protocol.

## Appendix 5: NHS board compliance per board for decision-making and timeliness

Board	Decisions made within 90 days*	Decision published on web within 14 days*
NHS A&A	23/23 (100%)	21/23 (91%)
NHS Borders	16/23 (70%)	16/23 (70%)
NHS D&G	22/23 (96%)	16/23 (70%)
NHS Fife	21/23 (91%)	23/23 (100%)
NHS FV	23/23 (100%)	11/23 (48%)
NHS Grampian	13/23 (57%)	19/23 (83%)
NHS GG&C	23/23 (100%)	23/23 (100%)
NHS Highland	18/23 (78%)	12/23 (52%)
NHS Lanarkshire	23/23 (100%)	23/23 (100%)
NHS Lothian	17/23 (74%)	23/23 (100%)
NHS Orkney	No Data Provided (as per NHS Grampian)	
NHS Shetland	No Data Provided (as per NHS Grampian)	
NHS Tayside	21/23 (91%)	6/23 (26%)
NHS WI	15/23 (65%)	0/23 (0%) – no data provided
<b>Average %</b> (across 12 boards)	<b>87%</b>	<b>70%</b>
<b>Median %</b> (across 12 boards)	<b>91%</b>	<b>77%</b>

\*on 23 medicines audited.



**Appendix 6: CMO(2012)1 guidance example list of SMC medicines and formulary decisions**

<b>SMC accepted medicine</b>	<b>Indication</b>	<b>Formulary decision &amp; rationale for non-inclusion</b>	<b>Date</b>
e.g. Everolimus (Afinitor)	Treatment of unresectable or metastatic, well- or moderately-differentiated neuroendocrine tumours of pancreatic origin in adults with progressive disease.	Not included - pending protocol	04/02/2013

## Appendix 7: Individual NHS board feedback

The below feedback is provided for each of the 12 NHS boards that provided complete data.

### NHS Ayrshire & Arran

NHS Ayrshire & Arran was 100% compliant with making their ADTC decisions on the 23 medicines within 90 days. 91% (21 out of 23) of the ADTC decisions on the 23 medicines were published on the board's website within 14 days of the ADTC decision being made.

The board's website was also easily to navigate and when using the website's main search function, and using the search terms noted in Appendix 2, links were provided to the NHS Ayrshire & Arran formulary. NHS Ayrshire & Arran also displayed their ADTC decisions in a manner consistent with the CMO guidance (see Appendix 6).

Of the 23 medicines, 17 were included on the NHS Ayrshire & Arran formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

ADTC decision categories	Number of medicines
1	15
2	2
3	0
4	2
5	1
6	3

### NHS Borders

NHS Borders was 70% compliant with making their ADTC decisions on the 23 medicines within 90 days. 70% (16 out of 23) of the ADTC decisions on the 23 medicines were published on the board's website within 14 days of the ADTC decision being made.

The board website failed to provide access to the NHS Borders formulary or links to the board's ADTC decisions.

Of the 23 medicines, 17 were included on the NHS Borders formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

ADTC decision categories	Number of medicines
1	17
2	0
3	1
4	1
5	0
6	4

## NHS Dumfries & Galloway

NHS Dumfries & Galloway was 96% compliant with making their ADTC decision on the 23 medicines within 90 days. 70% (16 out of 23) of the ADTC decisions on the 23 medicines were published on the board's website within 14 days of the ADTC decision being made.

NHS Dumfries & Galloway's website provided poor access to the board formulary and there was no readily available record of ADTC decisions. Using the search terms, detailed in Appendix 2, no relevant search results were found. NHS Dumfries & Galloway do have an external formulary website, which provides excellent access for patients and the public to local formulary decisions. However, a link was not provided on the board's main website.

Of the 23 medicine, 13 were included on the NHS Dumfries & Galloway formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

ADTC decision categories	Number of medicines
1	8
2	5
3	3
4	0
5	3
6	4

## NHS Fife

NHS Fife was 91% compliant with making their ADTC decisions on the 23 medicines within 90 days. 100% (23 out of 23) of the ADTC decisions on the 23 medicines were published on the board's website within 14 days of the ADTC decision being made. Both medicines that did not have a decision made on them within 90 days were cancer therapy medicines that required a decision from a regional cancer network on use before an ADTC decision could be made.

NHS Fife's website provided excellent access for patients and the public, with the first search term (from Appendix 2) providing a link to the ADTC section of the board website, which then provided links to the Fife joint formulary and ADTC decisions.

Of the 23 medicines, 21 were included on the NHS Fife formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

ADTC decision categories	Number of medicines
1	21
2	0
3	0
4	1
5	1
6	0

## **NHS Forth Valley**

NHS Forth Valley was 100% compliant with making their ADTC decisions on the 23 medicines within 90 days. 48% (11 out of 23) of the ADTC decisions on the 23 medicines were published on the board's website within 14 days of the ADTC decision being made. This discrepancy was due to publication dates not being provided for 11 of the 12 remaining medicines.

NHS Forth Valley's website provided very poor access for patients and the public with regards to access to ADTC decisions or the NHS Forth Valley formulary. None of the agreed search terms provided any relevant results.

Of the 23 medicines, 20 were included on the NHS Forth Valley formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

<b>ADTC decision categories</b>	<b>Number of medicines</b>
1	20
2	0
3	0
4	3
5	0
6	0

## **NHS Grampian (including NHS Orkney and NHS Shetland)**

NHS Grampian was 57% compliant with making their ADTC decisions on the 23 medicines within 90 days. 83% (19 out of 23) of the ADTC decisions on the 23 medicines were published on the board's website within 14 days of the ADTC decision being made. The formulary decision publication deadline was missed for four medicines and was related to server issues in all cases.

NHS Grampian's website had easy access for patients and the public to view the board's formulary or ADTC decisions. In most cases, when searching for the terms detailed in Appendix 2, links were provided to the NHS Grampian medicines management website; however these links did not always work. NHS Grampian indicated that they have been experiencing server issues in recent months, but are instigating a new system that should resolve these problems.

NHS Orkney and NHS Shetland are signed up to the decisions made by NHS Grampian's ADTC. NHS Shetland did not publish these ADTC decisions on their website. NHS Orkney provided good access to the NHS Grampian formulary and also displayed their ADTC decisions in a manner consistent with the CMO guidance (see Appendix 6). NHS Orkney and NHS Shetland did not return completed data collection forms, as they duplicate the decisions of NHS Grampian.

Of the 23 medicines, 15 were included on the NHS Grampian formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

<b>ADTC decision categories</b>	<b>Number of medicines</b>
1	14
2	1
3	0
4	2
5	6
6	0

### **NHS Greater Glasgow and Clyde**

NHS Greater Glasgow and Clyde was 100% compliant with making their ADTC decisions on the 23 medicines within 90 days. 100% (23 out of 23) of the ADTC decisions on the 23 medicines were published on the board's website within 14 days of the ADTC decision being made.

NHS Greater Glasgow and Clyde's website provided excellent access to ADTC decisions and the board's formulary. When searching the website (using the search criteria detailed in Appendix 2), links were provided to the medicines section of the board website where the above information could be found intuitively.

Of the 23 medicines, 20 were included on the NHS Greater Glasgow and Clyde formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

<b>ADTC decision categories</b>	<b>Number of medicines</b>
1	20
2	0
3	0
4	0
5	0
6	3

### **NHS Highland (and NHS Western Isles)**

NHS Highland was 78% compliant with making their ADTC decisions on the 23 medicines within 90 days. 52% (12 out of 23) of the ADTC decisions on the 23 medicines were published on the board's website within 14 days of the ADTC decision being made.

NHS Highland's website provided excellent access for patients and the public to view the board's formulary and their ADTC decisions. When using the search terms (detailed in Appendix 2), search results were returned for the pharmacy section of the website, which provided the formulary and links to the ADTC decision bulletins.

NHS Western Isles follows the ADTC decisions of NHS Highland. As NHS Highland was 74% compliant and NHS Western Isles was 65% compliant with making their ADTC decisions on the 23 medicines within 90 days, it can be concluded that there is a delay between NHS Highland's ADTC decisions being made and NHS Western

Isles making their subsequent decision. NHS Western Isles did not provide dates of when their ADTC decisions were published on their website and when searching the board website they did not provide access to the board's (or NHS Highland's) formulary or ADTC decision bulletins.

Of the 23 medicines, 15 were included on the NHS Highland (and NHS Western Isles) formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

<b>ADTC decision categories</b>	<b>Number of medicines</b>
1	15
2	0
3	0
4	8
5	0
6	0

### **NHS Lanarkshire**

NHS Lanarkshire was 100% compliant with making their ADTC decisions on the 23 medicines within 90 days. 100% (23 out of 23) of the ADTC decisions on the 23 medicines were published on the board's website within 14 days of the ADTC decision being made.

NHS Lanarkshire's website provided good access to the Lanarkshire formulary. However, this was only found after searching for 'ADTC' or 'formulary' on the board website. When using the other search terms detailed in Appendix 2, such as medicine, no relevant results were provided. Links were also provided to the NHS Lanarkshire ADTC bulletins. NHS Lanarkshire also displayed their ADTC decisions in a manner consistent with the CMO guidance (see Appendix 6).

Of the 23 medicines, 23 were included on the NHS Lanarkshire formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

<b>ADTC decision categories</b>	<b>Number of medicines</b>
1	23
2	0
3	0
4	0
5	0
6	0

### **NHS Lothian**

NHS Lothian was 74% compliant with making their ADTC decisions on the 23 medicines within 90 days. 100% (23 out of 23) of the ADTC decisions on the 23 medicines were published on the board's website within 14 days of the ADTC decision being made.

NHS Lothian’s website did not provide easy or intuitive access for patients and the public to view the board’s formulary or ADTC decisions. When searching the board’s website, using the search terms detailed in Appendix 2, one of the results provided was ‘links’; when selected, a series of links to external websites was provided; one of these was the Lothian Joint Formulary website. This link was not placed in a particularly intuitive location. However, when found, Lothian’s Joint Formulary website provided excellent information for patients and the public, detailing all local formulary decisions.

Of the 23 medicines, 13 were included on the NHS Lothian formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

<b>ADTC decision categories</b>	<b>Number of medicines</b>
1	11
2	2
3	0
4	3
5	3
6	4

### **NHS Tayside**

NHS Tayside was 91% compliant with making their ADTC decisions on the 23 medicines within 90 days. 26% (6 out of 23) of the ADTC decisions on the 23 medicines were published on the board’s website within 14 days of the ADTC decision being made.

NHS Tayside’s website does not have a search facility. Within the links section of the website, there is a link to the NHS Tayside ADTC website, which has links to the NHS Tayside formulary. There is also a link to the formulary and a new medicines search index, with accompanying text describing the local new medicines process. However, these links would not necessarily be apparent to patients.

Of the 23 medicines, 18 were included on the NHS Tayside formulary.

The breakdown of ADTC decisions on the 23 medicines is provided below.

<b>ADTC decision categories</b>	<b>Number of medicines</b>
1	18
2	0
3	0
4	3
5	0
6	2

## References

1. The Scottish Government. February 2012. CMO(2012)1: Guidance to Further Strengthen the Safe and Effective use of New Medicines Across the NHS in Scotland. [cited 2012 August 09]; Available from: [http://www.sehd.scot.nhs.uk/cmo/CMO\(2012\)01.pdf](http://www.sehd.scot.nhs.uk/cmo/CMO(2012)01.pdf)
2. The Scottish Government. February 2012. CEL 17 (2010): Introduction and Availability of Newly Licensed Medicines in the NHS in Scotland. [cited 2012 August 09]; Available from: [http://www.sehd.scot.nhs.uk/mels/CEL2010\\_17.pdf](http://www.sehd.scot.nhs.uk/mels/CEL2010_17.pdf)
3. The Scottish Government. March 2011. SGHD/CMO(2011)3: Implementing CEL 17 (2010): Introduction and Availability of Newly Licensed Medicines in the NHS in Scotland – Good Practice Guidance for NHS Board Management of Individual Patient Treatment Requests (IPTRs). [cited 2012 August 09]; Available from: [http://www.sehd.scot.nhs.uk/cmo/CMO\(2011\)03.pdf](http://www.sehd.scot.nhs.uk/cmo/CMO(2011)03.pdf)



### List of consultees

Professor Bill Scott, Chief Pharmaceutical Officer, Scottish Government

Dr Sara Davies, Public Health Consultant, Scottish Government

Mrs Laura McIver, Chief Pharmacist, Health Improvement Scotland

Scottish Association of Medical Directors

NHS Board Chief Pharmacists

The Health and Social Care Alliance

ABPI Scotland

SMC Patient and Public Involvement Group

Ms Joyce Mouriki and members of PPFFora and ADTC, Healthcare Improvement Scotland

NHS Greater Glasgow and Clyde Cancer Directorate

NHS Lothian Cancer Directorate Medicines Management Group

### References

1. Comparative analysis of the role and impact of Health Technology Assessment 2011:43 et seq(Charles River Associates, 2011)



**The Scottish  
Government**  
Riaghaltas na h-Alba

© Crown copyright 2013

You may re-use this information (excluding logos and images) free of charge in any format or medium, under the terms of the Open Government Licence. To view this licence, visit <http://www.nationalarchives.gov.uk/doc/open-government-licence/> or e-mail: [psi@nationalarchives.gsi.gov.uk](mailto:psi@nationalarchives.gsi.gov.uk).

Where we have identified any third party copyright information you will need to obtain permission from the copyright holders concerned.

ISBN: 978-1-78256-560-4 (web only)

The Scottish Government  
St Andrew's House  
Edinburgh  
EH1 3DG

Produced for the Scottish Government by APS Group Scotland  
DPPAS14222 (05/13)

Published by the Scottish Government, May 2013

w w w . s c o t l a n d . g o v . u k